UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2024

or

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

Commission File Number 001-36751

OCUGEN, INC.
(Exact name of registrant as specified in its charter)

Delaware	**04-3522315**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

11 Great Valley Parkway
Malvern, Pennsylvania 19355
(Address of principal executive offices, including zip code)
(484) 328-4701
(Registrant's telephone number, including area code)

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

Title of each class	**Trading symbol(s)**	**Name of each exchange on which registered**
Common Stock	**OCGN**	**The Nasdaq Stock Market LLC (The Nasdaq Capital Market)**

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

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

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

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

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

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

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

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

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

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

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

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

As of June 30, 2024, the last day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the common stock held by non-affiliates of the registrant was approximately $392.9 million, based upon the closing price of the registrant's common stock on June 30, 2024.

As of February 26, 2025, there were 292,014,470 outstanding shares of the registrant's common stock, $0.01 par value per share.

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report on Form 10-K ("Annual Report") incorporates certain information by reference from the registrant's proxy statement for the 2025 annual meeting of stockholders to be filed no later than 120 days after the end of the registrant's fiscal year ended December 31, 2024.

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Unless the context otherwise requires, references to the "Company," "we," "our," or "us" in this report refer to Ocugen, Inc. and its subsidiaries, and references to "OpCo" refer to Ocugen OpCo, Inc., the Company's wholly owned subsidiary.

DISCLOSURE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report and the documents incorporated by reference herein contain forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts contained in this Annual Report or the documents incorporated by reference herein, regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans, and objectives of management are forward-looking statements. These statements involve known and unknown risks, uncertainties, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "will," "would," or the negative of such terms and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are based on assumptions and expectations that may not be realized and are inherently subject to risks, uncertainties, and other factors, many of which cannot be predicted with accuracy and some of which might not even be anticipated.

The forward-looking statements in this Annual Report and the documents incorporated herein by reference include, among other things, statements about:

- our estimates regarding expenses, future revenues, and capital requirements, as well as the timing, availability of, and the need for, additional financing to continue to advance our product candidates;
- our activities with respect to OCU400, OCU410 and OCU410ST, including the results from our ongoing Phase 1/2 trials for OCU410 and OCU410ST, our ability to continue dosing patients for our Phase 3 trial for OCU400 for the treatment of retinitis pigmentosa ("RP");
- our ability to obtain additional funding from government agencies in the United States and/or other countries to continue the development of our inhaled mucosal vaccine platform;
- the uncertainties associated with the clinical development and regulatory approval of our product candidates including potential delays in the initiation, enrollment, and completion of current and future clinical trials;
- our ability to realize any value from our product candidates and preclinical programs being developed and anticipated to be developed, in light of inherent risks and difficulties involved in successfully commercializing products and the risk that our products, if approved, may not achieve broad market acceptance;
- our ability to comply with regulatory schemes and other regulatory developments applicable to our business in the United States and other countries;
- the performance of third-parties upon which we depend, including contract development and manufacturing organizations ("CMDOs"), suppliers, manufacturers, group purchasing organizations, distributors, and logistics providers;
- the pricing and reimbursement of our product candidates, if commercialized;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- developments relating to our competitors and our industry;
- our ability to obtain and maintain patent protection, or obtain licenses to intellectual property and defend our intellectual property rights against third-parties;
- our ability to maintain our relationships and contracts with our key collaborators and commercial partners and our ability to establish additional collaborations and partnerships;
- our ability to recruit and retain key scientific, technical, commercial, and management personnel and to retain our executive officers;
- matters relating to or arising from the restatement of our previously issued financial statements included in our Annual Report for the fiscal year ended December 31, 2022 as well as unaudited interim financial statements included in our Quarterly Reports on Forms 10-Q for each of the quarterly and year to date periods ended September 30, 2022, June 30, 2022, March 31, 2022, September 30, 2023, June 30, 2023, and March 31, 2023 (collectively, the "Previously Issued Financial Statements");
- our ability to comply with stringent United States and applicable foreign government regulations with respect to the manufacturing of pharmaceutical products, including compliance with current Good Manufacturing Practice ("GMP") regulations, and other relevant regulatory authorities; and

- the extent to which health epidemics and other outbreaks of communicable diseases, geopolitical turmoil, macroeconomic conditions, social unrest, political instability, terrorism, or acts of war could disrupt our business and operations, including impacts on our development programs, global supply chain, and collaborators and manufacturers.

We may not actually achieve the plans, intentions, or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions, and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report, particularly under "Risk Factors," that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations, investments, or other significant transactions we may make.

You should read this Annual Report and the documents that we incorporate by reference herein and have filed as exhibits to this Annual Report, completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Annual Report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements. We qualify all of our forward-looking statements by these cautionary statements. In addition, with respect to all of our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

Solely for convenience, tradenames and trademarks referred to in this Annual Report appear without the ® or ™ symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or that the applicable owner will not assert its rights, to these tradenames or trademarks, as applicable. All tradenames, trademarks, and service marks included or incorporated by reference in this Annual Report are the property of their respective owners. The name NeoCart (defined below) has not been evaluated or cleared by the FDA.

PART I

Item 1. Business.

OVERVIEW

We are a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics and vaccines that improve health and offer hope for patients across the globe.

Our technology pipeline includes:

- **Modifier Gene Therapy Platform** — Based on the use of nuclear hormone receptors ("NHRs"), we believe our modifier gene therapy platform has the potential to address many retinal diseases, including rare genetic diseases such as RP (OCU400), with a gene-agnostic approach. We also believe our modifier gene therapy platform has the potential to address multifactorial retinal diseases including dry age-related macular degeneration ("dAMD"), which affects millions of patients in the United States alone, using OCU410, and Stargardt disease, which is a rare genetic disease, using OCU410ST. We are actively recruiting patients in the United States and Canada in the Phase 3 liMeliGhT clinical trial for OCU400 for the treatment of RP and are on track to complete enrollment in the first half of 2025. In January 2025, we announced positive two year safety and efficacy data across multiple mutations from the Phase 1/2 clinical trial of OCU400. In February 2025, we announced that the European Commission (EC) has provided a positive opinion from the European Medicines Agency (EMA) Committee for Advanced Therapies for OCU400 Advanced Therapy Medicinal Product (ATMP) classification. OCU410 completed dosing in Phase 2 of the Phase 1/2 ArMaDa clinical trial for the treatment of geographic atrophy ("GA"), an advanced form of dAMD. Positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the OCU410 Phase 1/2 ArMaDa clinical trial included: no drug-related serious adverse events ("SAEs"), reduced lesion growth, preservation of retinal tissue, and—most importantly—there was a positive effect on the functional visual measure of low luminance visual acuity ("LLVA"). In October 2024, the Data and Safety Monitoring Board ("DSMB") for the OCU410ST GARDian clinical trial approved enrollment for the second phase of the Phase 1/2 clinical trial. In February 2025, we announced that alignment has been reached with the FDA to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a BLA submission. Additionally, the European Medicines Agency ("EMA") granted orphan medicinal product designation ("OMPD") for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, RP 19 ("RP19"), and cone-rod dystrophy 3 ("CORD3"). Both OCU410 and OCU410ST studies have completed the low, medium, and high dose cohorts in the Phase 1 part of the Phase 1/2 trials to date, with OCU410 completing dosing in the Phase 2 part of the Phase 1/2 trials.

- **Novel Biologic Therapy for Retinal Diseases** — OCU200 is a novel fusion protein consisting of two human proteins, tumstatin and transferrin. OCU200 possesses unique features which potentially enable it to treat vascular complications of diabetic macular edema ("DME"), diabetic retinopathy ("DR"), and wet age-related macular degeneration ("AMD"). Tumstatin is the active component of OCU200 and binds to integrin receptors, which play a crucial role in disease pathogenesis. Transferrin is expected to facilitate the targeted delivery of tumstatin into the retina and choroid and potentially help increase the interaction between tumstatin and integrin receptors. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and currently we are actively recruiting patients.
- **Regenerative Medicine Cell Therapy Platform** — Our Phase 3-ready regenerative cell therapy platform technology, which includes NeoCart (autologous chondrocyte-derived neocartilage), is being developed for the repair of knee cartilage injuries in adults. We received concurrence from the FDA on the confirmatory Phase 3 trial design and have completed renovating an existing facility into a current GMP facility to support clinical study and initial commercial launch. This facility is needed to generate patient-specific NeoCart implant from chondrocytes derived from knee biopsy. We intend to initiate the Phase 3 trial contingent on adequate availability of funding.
- **Inhaled Mucosal Vaccine Platform** — Our next-generation, inhaled mucosal vaccine platform includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine. We have completed IND-enabling studies and GMP manufacturing of clinical trial material for OCU500. In January 2025, we announced that the Investigational New Drug (IND) application is in effect and the National Institute of Allergy and Infectious Diseases ("NIAID") intends to initiate a Phase 1 clinical trial in the second quarter of 2025. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

Modifier Gene Therapy Platform

We are developing a modifier gene therapy platform designed to fulfill unmet medical needs related to retinal diseases, including inherited retinal diseases ("IRDs"), such as RP, Stargardt disease; and multifactorial diseases such as dAMD. Our modifier gene therapy platform is based on the use of NHRs, which have the potential to achieve homeostasis — the basic biological processes in the retina to restore a healthy state from a diseased state. Unlike single gene replacement therapies, which only target one genetic mutation, our modifier gene therapy platform, through its use of NHRs, represents a unique, gene-agnostic approach designed to address not just the mutated gene but provide a molecular "reset" of health and survival of gene networks. OCU400, our lead product candidate in our modifier gene therapy platform, has received Orphan Drug Designation ("ODD") from the FDA for RP and LCA, a regenerative medicine advanced therapy ("RMAT") designation for the treatment of RP associated with *NR2E3* and rhodopsin ("*RHO")* mutations from the FDA, and OMPD from the European Commission ("EC"), based on the recommendation of the EMA, for RP and LCA. These broad ODD, RMAT, and OMPD designations further support the broad (gene-agnostic) therapeutic potential of OCU400 to treat RP associated with mutations in multiple genes.

The OCU400 Phase 3 liMeliGhT clinical trial is currently underway, with enrollment on track to meet our target submission of a Biologics License Application ("BLA") and Marketing Authorization Application ("MAA") in mid-2026.

In August 2024, we received notification from the FDA that we could begin our expanded access program ("EAP") for the treatment of adult patients with RP with OCU400. This program is available for patients with early, intermediate to advanced RP with at least minimal retinal preservation who may benefit from the mechanism of action of OCU400 prior to approval of the BLA.

We also received approval from Health Canada to initiate a Phase 3 trial for OCU400 for the treatment of RP. The Health Canada trial will run in parallel with the United States FDA trial, expediting the ability to potentially provide a gene-agnostic treatment option to approximately 110,000 patients in the United States and Canada.

In January 2025, we announced positive two-year long-term data across multiple mutations from the Phase 1/2 clinical trial of OCU400, which demonstrated a durable and statistically significant (p=0.005) improvement in visual function (LLVA) in all evaluable treated subjects at two years when compared to untreated eyes. 100% (10/10) of treated evaluable subjects demonstrated improvement or preservation in visual function compared to untreated eyes. Also, treated eyes with multiple mutations and *RHO* subjects demonstrated a statistically significant (p=0.005) improvement in visual function when compared to untreated eyes. Treated eyes in *RHO* subjects (who meet Intent-to-treat criteria) for OCU400 Phase 3 trial demonstrated a statistically significant improvement (p=0.01, N=4) with around two-line gain in visual function (LLVA) at two years when compared to untreated eyes.

In February 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU400 ATMP classification. ATMP classification is granted to medicines that can offer groundbreaking opportunities for the treatment of disease and accelerates the regulatory review timeline of this potential one-time gene therapy for life.

OCU410 and OCU410ST are being developed utilizing the nuclear receptor gene RAR-related orphan receptor A ("*RORA")*, for the treatment of GA secondary to dAMD and Stargardt disease, respectively. OCU410 is a potential one-time, curative therapy with a single sub-retinal injection. OCU410 targets multiple pathways associated with AMD pathogenesis, in contrast to products currently approved or under development that treat only one cause of GA, require multiple injections per year, and have safety considerations. OCU410ST has received ODD from the FDA for the treatment of *ABCA4*-associated retinopathies, including Stargardt disease. OCU410ST has also received OMPD from the EMA for the treatment of *ABCA4*-associated retinopathies, including Stargardt disease, RP19, and CORD. In March 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU410 and OCU410ST ATMP classification.

OCU410 has completed dosing in the Phase 2 of the Phase 1/2 ArMaDa clinical trial. OCU410ST has completed Phase 1 of the Phase 1/2 GARDian clinical trial, and the DSMB has approved enrollment for the second phase of the Phase 1/2 GARDian clinical trial.

In November 2024, we announced positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the Phase 1/2 ArMaDa clinical trial for GA. The key findings included: no drug-related SAEs, reduced lesion growth, preservation of retinal tissue, and a positive effect on the functional visual measure of LLVA. In February 2025, we announced dosing has been completed in Phase 2 of the OCU410 ArMaDa clinical trial. Phase 2 was a randomized expansion phase in which 51 subjects were randomized in a 1:1:1 ratio to either one of two OCU410 dose groups or to an untreated control group.

In October 2024, we announced that the DSMB had approved proceeding to Phase 2 of the OCU410ST GARDian trial using high and medium doses. In November 2024, we announced preliminary efficacy and safety data from evaluable subjects at six

months from the Phase 1 dose-escalation portion of the Phase 1/2 OCU410ST GARDian clinical trial, which demonstrated an 84% reduction in atrophic lesion growth in treated eyes versus the corresponding untreated eyes.

In February 2025, we announced that alignment has been reached with the FDA to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a BLA submission. The Phase 2/3 clinical trial will randomize 51 subjects, 34 of whom will receive a single, subretinal, 200-µL injection of OCU410ST at a concentration of 1.5×10^{11} vector genomes (vg)/mL in the eye with worse visual acuity, and 17 of whom will serve as untreated controls. The primary endpoint in the clinical trial is change in atrophic lesion size. Secondary endpoints include visual acuity as measured by best corrected visual acuity ("BCVA") and LLVA compared to untreated controls. One-year data will be utilized for the BLA filing.

Novel Biologic Therapy for Retinal Diseases

We are developing OCU200, which is a novel fusion protein containing parts of human transferrin and tumstatin. OCU200 is designed to treat DME, DR, and wet AMD. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and we are actively recruiting patients for the Phase 1 clinical trial. The OCU200 Phase 1 clinical trial is a multicenter, open-label, dose-escalation study to assess drug safety via intravitreal injection in three cohorts: low dose (0.025 mg), medium dose (0.05 mg), and high dose (0.1 mg). All subjects will receive a total of two intravitreal injections of OCU200 six weeks apart. Patient follow-up will take place up to three months after the last injection.

Regenerative Medicine Cell Therapy Platform

NeoCart is a Phase 3-ready, regenerative cell therapy technology that combines breakthroughs in bioengineering and cell processing to enhance the autologous cartilage repair process. NeoCart is a three-dimensional tissue-engineered disc of new cartilage that is manufactured by growing the patient's own chondrocytes, the cells responsible for maintaining cartilage health. Current surgical and nonsurgical treatment options for knee cartilage injuries in adults are limited in their efficacy and durability. In prior clinical studies, Phase 2 and Phase 3, NeoCart has shown potential to accelerate healing, reduce pain, and provide regenerative native-like cartilage strength with durable benefits post transplantation. NeoCart was shown to be generally well-tolerated and demonstrated greater clinical efficacy than microfracture surgery at two years after treatment. Based on this clinical benefit, the FDA granted a RMAT designation to NeoCart for the repair of full-thickness lesions of knee cartilage injuries in adults. Additionally, we received concurrence from the FDA on the confirmatory Phase 3 trial design where chondroplasty will be used as a control group. We have completed renovating an existing facility into a GMP facility in accordance with the FDA's regulations in support of NeoCart manufacturing for personalized Phase 3 trial material. We intend to initiate the Phase 3 trial contingent on adequate availability of funding.

Inhaled Mucosal Vaccine Platform

We are party to an exclusive license agreement (as amended, "WU License Agreement") with The Washington University in St. Louis ("Washington University"), pursuant to which we licensed the rights to develop, manufacture, and commercialize a mucosal COVID-19 vaccine for the prevention of COVID-19 in the United States, Europe, Japan, South Korea, Australia, China, and Hong Kong (the "Mucosal Vaccine Territory"). In addition, we internally developed technology related to the flu and COVID-19's vaccine design and filed intellectual property. We are developing a next-generation, inhalation-based mucosal vaccine platform based on a novel ChAd vector, which includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine. Our inhaled mucosal vaccine platform is driven by our conviction to serve a major public health concern, which requires the endorsement and support of government funding in order to develop and ultimately commercialize our vaccine candidates. As these vaccine candidates are being developed to be administered via inhalation, we believe they have the potential to generate rapid local immune response in the upper airways and lungs, where viruses enter and infect the body. We believe this novel delivery route may help reduce or prevent infection and transmission as well as provide protection against new virus variants. In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. OCU500 will be tested via two different mucosal routes, inhalation and intranasal delivery. In January 2025, we announced that the IND application is in effect to initiate the Phase 1 clinical trial of OCU500. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

OUR STRATEGY

We are developing novel solutions to medical challenges and approaching healthcare innovation with purpose and agility to deliver new options for people facing serious diseases and conditions. We believe our product candidates have the potential to

treat blindness diseases, treat serious conditions such as articular cartilage lesions, and reduce transmission or prevent respiratory diseases such as the flu and COVID-19. Key elements of the strategy we employ to accomplish this mission include:

- ***Continuing to advance our modifier gene therapy platform into and through clinical development.***

  We are developing our modifier gene therapy platform, inclusive of OCU400, OCU410, and OCU410ST, for the treatment of multiple IRDs, including RP, Stargardt disease and multifactorial diseases such as dAMD and GA. We are actively recruiting patients in the United States and Canada in the Phase 3 liMeliGhT clinical trial for OCU400 for the treatment of RP. Both OCU410 and OCU410ST studies have completed the low, medium, and high dose cohorts in the Phase 1 part of the Phase 1/2 trials to date, with OCU410 completing dosing in the Phase 2 part of the Phase 1/2 trials.

- ***Expanding and exploring partnerships with current and future key collaborators and commercial partners to maximize patient access, global reach, and the value of our product candidates.***

  We intend to explore strategic licensing, acquisition, and collaboration opportunities with qualified partners to maximize the potential benefit of our product candidates for patients globally and to expand our product candidate pipeline to support our future growth.

- ***Obtaining government funding to advance our vaccine programs towards commercialization.***

  We are developing a next-generation, inhalation-based mucosal vaccine platform based on a novel ChAd vector, which includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine. In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. NIAID intends to initiate a Phase 1 clinical trial in the second quarter of 2025. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

- ***Advancing the clinical development of our regenerative medicine platform towards market authorization and developing in-house manufacturing capability.***

  We are developing NeoCart, our regenerative medicine platform technology for the repair of knee cartilage injuries in adults. We have received concurrence from the FDA on the confirmatory Phase 3 clinical trial design. We have completed renovating an existing facility into a current GMP facility in accordance with the FDA's regulations in support of NeoCart manufacturing for Phase 3 clinical trial material. We intend to initiate the Phase 3 trial contingent on adequate availability of funding.

COMPETITIVE STRENGTHS

Our key competitive strengths include:

- ***Platform Technologies.*** Our cutting-edge modifier gene therapy platform provides a competitive advantage compared to traditional approaches for gene therapy, such as gene augmentation, replacement or editing. This platform technology utilizes NHR genes, master regulators inside cells, which promotes homeostasis – a balanced physiological state within cells. Our OCU400 modifier gene therapy product candidate can address >100 gene mutations associated with RP disease, in contrast, the traditional approach can address only one gene mutation at a time with single product. This technology has versatility in addressing various diseases of the retina with potential for expansion into other disease areas. Our OCU410 modifier gene therapy product candidate has potential to treat both genetic (Stargardt disease) as well as complex multifactorial disease (AMD). Our 3D tissue engineering platform technology utilizes state-of-the-art bioreactor which can be used to produce native cartilage-like tissues for implantation. Our first product candidate based on this platform technology, NeoCart, is an autologous cartilage tissue–engineered implant designed for use in repair of articular cartilage injuries in the knee. In contrast to the approved cell-matrix based product, NeoCart showed rapid healing and durable benefit in clinical studies. Our inhaled vaccine platform technology utilizes combination of ChAd vector technology and inhalation delivery technology, which can be utilized for vaccine development for various respiratory diseases. The OCU500 vaccine series is based on a novel ChAd platform designed to reduce transmission and protect against new variants with long-term durability. The inhaled method offers the potential for broad, durable protection from severe disease and reduction in transmission.

- ***Experienced Management Team and Esteemed Scientific and Business Advisory Boards.*** Our management team and key advisors have extensive experience with a proven track record of success in developing, launching, and managing the life cycle of biopharmaceuticals and vaccines at leading pharmaceutical and biotechnology companies. Our retina and vaccine scientific advisory boards are composed of leading academic and industry experts with extensive experience in the ocular and infectious disease fields. Our business advisory board members have been selected based on their extensive professional backgrounds and proven track record of creating partnerships among the public and

private sector. We believe that the experience of our management team, our scientific advisory board members, business advisory board, and our broad network of relationships with leaders within the industry and the medical community provides us with insight into the identification of product candidate opportunities as well as supports us in advancing the development and commercialization of our product candidates.

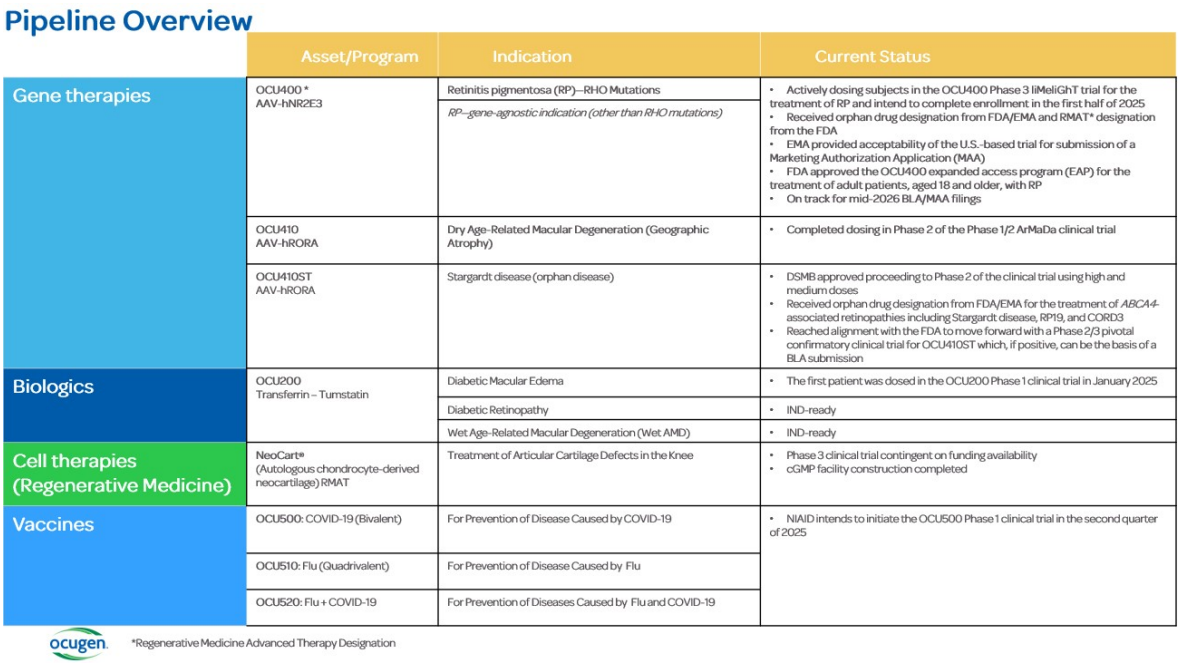
- ***Key Partnerships and Internal Capabilities.*** We have established a partnership with CanSino Biologics, Inc. ("CanSinoBIO") for our modifier gene therapy platform. CanSinoBIO has state-of-the-art facilities and proven expertise in the gene therapy field, which is critical to advancing our gene therapy product candidates into and through clinical trials as well as accelerating development timelines, reducing our associated costs, and increasing the reliability of our product candidate manufacturing. We have a state-of-the-art R&D center to support innovation and development of our product pipeline through commercialization. Our renovated GMP facility is designed to support manufacturing of cell and gene therapy-based products for clinical development as well as commercial launch. Our R&D, clinical, regulatory, quality and manufacturing teams consist of experienced, highly qualified researchers and industry professionals from top institutional and leading companies, which help drive pipeline development with greater efficiency.

- ***Product Designations.*** OCU400 has received ODD from the FDA for RP and LCA as well as been granted a RMAT designation from the FDA for the treatment of RP associated with *NR2E3* and *RHO* mutations. OCU400 has also received OMPD from the EC, based on the recommendation of the EMA, for RP and LCA. These designations demonstrate the potential broad-spectrum application of OCU400, through its use of NHRs, to treat the more than 125 genes associated with RP and LCA with one product rather than developing individual treatments for each gene mutation. OCU410ST has received ODD from the FDA for the treatment of *ABCA4*-associated retinopathies, including Stargardt disease. OCU410ST has also received OMPD from the EMA, for the treatment of *ABCA4*-associated retinopathies including Stargardt, RP19 and CORD3 diseases. NeoCart, our regenerative medicine cell therapy technology, was granted RMAT designation from the FDA for the repair of knee cartilage injuries in adults. The RMAT designation was created to expedite the development and review of regenerative medicine therapies intended to treat, modify, reverse, or cure a serious condition.

- ***Licensing and Development Arrangements and Intellectual Property Portfolio.*** We have licensing and development arrangements with leading companies, academic institutions, and medical institutions that cover our product candidates. These licensing and development arrangements include the licensing agreement with The Schepens Eye Research Institute, Inc. ("SERI"), an affiliate of Harvard Medical School, through which we acquired the technology used in our modifier gene therapy platform as well as access to technologies for other NHR genes, the license agreement with Purpose Co., Ltd. ("Purpose") relating to NeoCart, the WU License Agreement with Washington University with respect to mucosal COVID-19 vaccines in the Mucosal Vaccine Territory, and the license agreement with the University of Colorado ("CU") pursuant to which we acquired rights to the transferrin-tumstatin fusion protein technology used in our OCU200 product candidate. As of February 24, 2025, our global intellectual property portfolio contains 63 patents and 30 pending patent applications related to composition of matter, pharmaceutical compositions, methods of use for our product candidates, and other proprietary technology including those under our licensing and development arrangements. We will leverage these domestic and global partnerships and our intellectual property portfolio to advance our near- and long-term product pipeline opportunities.

OUR PRODUCT CANDIDATE PIPELINE

Our product candidate pipeline is summarized in the following chart:



OUR MODIFIER GENE THERAPY PLATFORM AND GENE THERAPY PRODUCT CANDIDATES

We are developing our modifier gene therapy platform, inclusive of OCU400, OCU410, and OCU410ST for the treatment of multiple IRDs, such as RP, Stargardt disease and multifactorial diseases such as dAMD and GA. Our modifier gene therapy platform is a cutting-edge technology licensed from SERI, an affiliate of Harvard Medical School, and involves the targeted delivery and expression of one or more NHRs in the disease tissues and is designed to introduce a functional gene to modify the expression of multiple genes and gene-networks, which potentially enables it to address multiple retinal diseases with one product.

Overview of RP and Current Treatment Options

IRDs are caused by genetic mutations that are passed down within families and lead to progressive disease, severe visual impairment, and blindness. They are a diverse disease class with large phenotypic and genetic heterogeneity. IRDs are a common cause of irreversible blindness due to retinal cell degeneration. Treating these conditions has been a significant challenge due to the sheer volume of potential therapeutic gene targets. Gene replacement therapy is a promising approach to provide sustained restoration of normal retinal function for a mutated gene, but such therapies can only address one gene at a time, limiting their potential therapeutic use. Developing a custom gene therapy for each of the more than 100 mutated genes linked to RP would not only be expensive but also may not be possible due to size, class, or localization that will impact delivery of the gene. Not all genes and disease expressions are amenable to gene therapy. For example, the genetic mutations of approximately 40% of RP patients remain unknown with few or no known therapeutic options available. Modifier gene therapy to ameliorate multiple forms of RP without requiring knowledge of the mutated gene, may provide a potentially robust and feasible treatment for RP.

RP is the most common IRD involving photoreceptors and the retinal pigment epithelium ("RPE"). RP is a group of rare, genetic disorders that involve a breakdown and loss of cells in the retina. RP affects approximately 310,000 individuals in the

United States, Europe, and Canada. In RP, progressive retinal degeneration starts in the mid-periphery and advances toward the macula and the fovea. The fovea is the part of the retina that is responsible for sharp central vision. Common symptoms of RP include difficulty seeing in poor lighting or in the dark, loss of central vision or side (peripheral) vision, and difficulty reading print and deciphering detailed images. RP is associated with over 100 mutated genes that affect about 1.6 million to 2 million individuals worldwide. RP is heterogeneous and varies greatly in age of onset, rate of progression, and even genetic etiology, yet all of the mutations lead to a common pathology of photoreceptor cell degeneration.

There is currently no approved treatment that slows or stops the progression of multiple forms of RP. Proposed treatments for RP include gene-replacement therapy, retinal implant devices, retinal transplantation, stem cells, vitamin therapy, and other pharmacological treatments. While gene-replacement therapies may provide a new functional gene, they do not necessarily eliminate the underlying genetic defect, which may still cause stress and toxic effects. Therefore, the development of gene specific replacement therapy is highly challenging, especially when multiple and unknown genes are involved.

OCU400 for IRDs

OCU400 is our first product candidate being developed with our modifier gene therapy platform. OCU400 has the potential to restore retinal integrity and function across a range of genetically diverse IRDs. OCU400 consists of a functional copy of the retina-specific NHR gene, *NR2E3*, delivered to target cells in the retina using an AAV5 vector that has the potential to be used as a gene therapeutic not only for the treatment of retinal diseases associated with mutations in genes such as *NR2E3*, *RHO*, and *PDE6ß*, but also other gene mutations associated with RP. As a potent modifier gene, expression of *NR2E3* may help reset retinal cell homeostasis, metabolism, and visual cycle function (**Figure 9**). OCU400 has received ODD for RP and LCA, a RMAT designation to OCU400 for the treatment of RP associated with *NR2E3* and *RHO*, and OMPD from the EC, based on the recommendation of the EMA, for RP and LCA. We believe these broad ODD, RMAT, and OMPD designations demonstrate that OCU400 has the potential to be a broad-spectrum therapeutic to treat RP. These ODD, RMAT, and OMPD designations represent gene-agnostic broad coverage for RP and is not mutation-specific designations.

Figure 9: Mechanism of our modifier gene therapy.

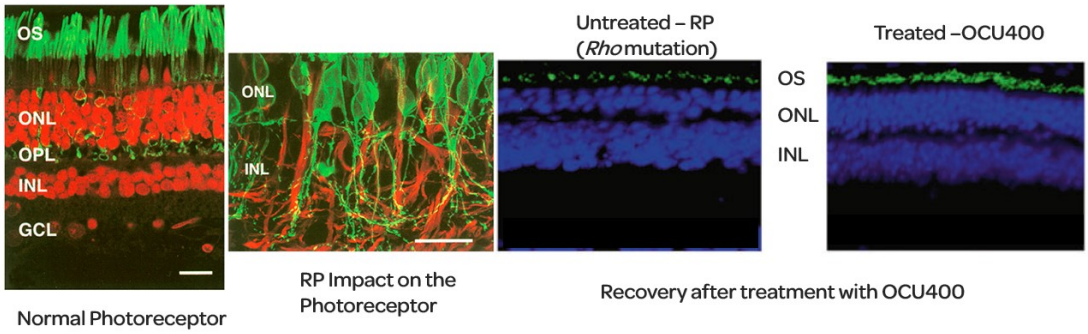


Figure 9 *demonstrates the mechanism of our modifier gene therapy. In single-gene replacement therapies such as gene augmentation, only the non-functional gene is targeted. and accordingly, this therapy cannot improve a multitude of disease-causing genetic defects. In our modifier gene therapy platform, a functional gene of the retina-specific NHR gene, NR2E3, is introduced to modify the expression of many genes and gene networks and restore homeostasis.*

The first patient has been dosed in our Phase 3 clinical trial for OCU400 for the treatment of RP. We received notification from the FDA that we could begin our EAP for the treatment of adult patients with RP with OCU400. We also received approval from Health Canada to initiate a Phase 3 trial for OCU400 for the treatment of RP. In February 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU400 ATMP classification. The Phase 3 clinical trial is on track to complete enrollment in the first half of 2025, file the BLA and MAA in mid-2026, and pursue commercialization in 2027, if approved.

Modifier Gene Therapy Platform Based on the Use of NHRs

NHRs are intracellular receptors that regulate gene expression, acting as master regulator genes in the retina. NHRs play a vital role in regulating retinal cell development, maturation, metabolism, visual cycle function, survival, and maintaining the cellular

and molecular homeostasis in retinal tissues. Our modifier gene therapy platform is designed to target NHRs to potentially provide therapeutic benefit to patients suffering from genetically diverse IRDs. The use of genetic modifiers represents a broadened means of potentially treating a variety of retinal degenerative diseases, as compared to single-gene replacement therapy. While single-gene replacement therapies have shown tremendous promise in rare retinal diseases, they are highly specific and cannot improve a multitude of disease-causing genetic defects. Our modifier gene therapy platform has the potential to restore retinal integrity and function across a range of genetically diverse IRDs and other degenerative retinal diseases providing us with significant potential long-term value.

Our modifier gene therapy platform encompasses the targeted delivery and expression of certain NHRs that are expressed naturally in retinal tissue. Preclinical studies have shown that *NR2E3*, a member of the NHR family, is a dual activator and repressor that, with other transcription factors, modulates cell fate and differentiation of rod and cone photoreceptor cells, specialized cells for detecting light, in the eye. Disease outcome is a result of a primary mutation as well as modifier alleles. *NR2E3* is a master regulator of several key pathways in retinal development and function. *NR2E3* potentially prevents and rescues degenerating retina by resetting the homeostatic state of key gene networks in the presence of a primary mutation.

The delivery of *Nr2e3* in a mouse lacking a functional *Nr2e3* gene restored the retina structure and function. We believe that *NR2E3* may partially or fully rescue photoreceptors from degeneration in patients with IRDs and improve patients' vision. It was also demonstrated preclinically that *RORA* offers a protective allele in AMD where the loss of photoreceptor cells leads to blindness. NR2E3 regulates the expression of both Nuclear Receptor Subfamily 1 Group D Member 1 ("*NR1D1*") and *RORA*. Thus, the nuclear receptors work in overlapping networks to modulate normal retinal development and function. These receptors impact gene expression of hundreds of genes and numerous networks and, as such, may be potent modifiers of retinal disease and degeneration.

OCU400 Phase 3 Clinical Study Overview

In April 2024, the FDA cleared our IND amendment to initiate a Phase 3 liMeliGhT clinical trial of OCU400 for RP. We are actively recruiting patients in the United States and Canada in the Phase 3 liMeliGhT clinical trial for OCU400 for the treatment of RP and are on track to complete enrollment in the first half of 2025. OCU400 is the first gene therapy program to enter Phase 3 with a broad RP indication. This Phase 3 trial will enroll 150 subjects, distributed 1:1 into two separate arms (*RHO*: N=75, and Gene Agnostic: N=75). In each arm subjects will be further randomized into 2:1 ratio to treated and untreated control groups. Subjects will be followed for a year after dosing for primary end point analyses. In the Phase 1/2 OCU400 clinical trial a MLMT scale was the primary functional endpoint. For the Phase 3 OCU400 clinical trial, an updated mobility course will be used, Luminance Dependent Navigation Assessment ("LDNA") that includes a wider range of light intensity (0.04-500 Lux) and Lux Levels (0-9) with a uniform correlation between Lux level and Lux intensity. More than 60% of intent-to-treat (ITT) patients from the Phase 1/2 clinical trial (*RHO* and *NR2E3*) meet the responder criteria (Responder ≥ 2 Lux level improvement) for Phase 3.

OCU400 Phase 1/2 Clinical Study Results

In January 2025, we announced positive two year long term data across multiple mutations from the Phase 1/2 clinical trial of OCU400, which demonstrated a durable and statistically significant (p=0.005) improvement in visual function (LLVA) in all evaluable treated subjects at two years when compared to untreated eyes. 100% (10/10) of treated evaluable subjects demonstrated improvement or preservation in visual function compared to untreated eyes. Also, treated eyes with multiple mutations and *RHO* subjects demonstrated a statistically significant (p=0.005) improvement in visual function when compared to untreated eyes. Treated eyes in *RHO* subjects (who meet Intent-to-treat criteria) for OCU400 Phase 3 trial demonstrated a statistically significant improvement (p=0.01, N=4) with around two line gain in visual function (LLVA) at two years when compared to untreated eyes.

In the Phase 1/2 OCU400-101 study, a total of 22 subjects aged nine to 77 years, male and female, received OCU400 subretinal injection in three dose levels of up to 300 µL. All subjects had a confirmed molecular diagnosis of either a biallelic autosomal recessive *NR2E3* mutations, or autosomal dominant *NR2E3* mutation, or autosomal dominant *RHO* mutations or *CEP290* mutation. In continuation of the preliminary analyses update, we announced an update for 18 participants. The positive trial update demonstrated that OCU400 continued to be generally safe and well-tolerated in subjects across different mutations and dose levels. 89% of participants demonstrated preservation or improvement in the treated eye either on BCVA or LLVA or MLMT scores from baseline. 78% of participants demonstrated preservation or improvement in the treated eyes in MLMT

scores from baseline. 80% of *RHO* mutation subjects experienced either preservation or improvement in MLMT scores from baseline.

Overview of dAMD and Stargardt Disease and Current Treatment Options

dAMD is attributed to the thinning of the macula of the retina, which leads to impairment and loss of central vision. The macula is the part of the retina responsible for clear vision in one's direct line of sight. dAMD is characterized by the thickening and loss of normal architecture within the Bruch's membrane, lipofuscin accumulation in the RPE, and drusen formation beneath the RPE in the Bruch's membrane. These deposits consist of complement components, other inflammatory molecules, lipids, lipoproteins B and E, and glycoproteins. Common risk factors for dAMD include genetics, smoking, nutrition and vitamin deficiency, and heart disease. dAMD, which affects over 266 million individuals worldwide, involves the slow deterioration of the retina with submacular drusen (small white or yellow dots on the retina), atrophy, loss of macular function, and central vision impairment. Common symptoms of dAMD include visual distortions, reduced central vision in one or both eyes, increased difficulty adapting to low levels of light, and a well-defined blind spot in one's field of vision. GA is an advanced form of dAMD that affects approximately two to three million people in the United States and Europe and eight million people worldwide. GA involves several biological factors and pathways, including lipid metabolism, oxidative stress, inflammation, and activation of the complement system. The currently approved treatments for GA only focus on the complement system with limited treatment benefits.

Similarly, Stargardt disease is a rare genetic eye disorder that causes retinal degeneration and ultimately leads to loss of central vision. It is the most common form of inherited macular degeneration, affecting approximately 0.1 million people in the United States and Europe. Stargardt disease happens when lipofuscin, a fatty yellow pigment, accumulates on the macula, which leads to the degeneration of the photoreceptor cells in the macula and ultimately leads to progressive central vision loss. The photoreceptor cells convert light into electrical signals, which are then sent to the brain where they are processed to create the images we see. Stargardt disease is usually caused by mutations in the *ABCA4* gene and is inherited in an autosomal recessive manner. This gene affects how a person's body uses vitamin A. The body uses vitamin A to make cells in the retina. Common symptoms of Stargardt disease include gray, black, or hazy spots in one's central vision, sensitivity to light, increased time for eyes to adjust between light and dark places, color blindness, and gradual central vision loss in both eyes. Currently no treatment options exist to address all four disease pathways related to dAMD or reverse or slow the progression of Stargardt disease, and accordingly, there remains a significant unmet medical need for these ocular diseases.

OCU410 and OCU410ST for the Treatment of Dry AMD and Stargardt Disease

We are developing OCU410 and OCU410ST for the treatment of dAMD and Stargardt disease, respectively. OCU410 and OCU410ST utilize an AAV delivery platform for the retinal delivery of the *RORA* gene. *RORA* regulated gene networks are relevant in the treatment of dAMD and Stargardt disease. *RORA* reduces oxidative stress, limits lipofuscin deposits, reduces chronic inflammation, regulates complement activation, and improves choroidal blood flow. Gene variants of the *ABCA4* gene are associated with both AMD and Stargardt disease. Stargardt disease is usually caused by mutations in the *ABCA4* gene. This gene transports oxidized retinol compounds from photoreceptors to RPE cells for detoxification. In mice models, *ABCA4* -/- displayed low levels of CD59. A cell-surface glycoprotein, CD59, prevents the formation of the complement membrane attack complex. Our OCU410 and OCU410ST programs have completed the low, medium, and high dose cohorts in the Phase 1 part of the Phase 1/2 trials to date. OCU410 completed dosing in the Phase 2 part of the Phase 1/2 ArMaDa clinical trial. OCU410ST has completed Phase 1 of the Phase 1/2 GARDian clinical trial, and the DSMB has approved enrollment for the second phase of the trial.

In February 2025, we announced that alignment has been reached with the FDA to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a BLA submission. The Phase 2/3 clinical trial will randomize 51 subjects, 34 of whom will receive a single, subretinal, 200-µL injection of OCU410ST at a concentration of 1.5×10^{11} vector genomes (vg)/mL in the eye with worse visual acuity, and 17 of whom will serve as untreated controls. The primary endpoint in the clinical trial is change in atrophic lesion size. Secondary endpoints include visual acuity as measured by best corrected visual acuity (BCVA) and LLVA compared to untreated controls. One-year data will be utilized for the BLA filing. In March 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU410 and OCU410ST ATMP classification.

NOVEL BIOLOGIC PRODUCT CANDIDATE FOR RETINAL DISEASES

We are developing OCU200, which is a novel fusion protein containing parts of human transferrin and tumstatin. OCU200 is designed to treat DME, DR, and Wet AMD. We have completed the technology transfer of manufacturing processes to our CDMO and have produced trial materials to initiate a Phase 1 trial. The Phase 1 clinical trial will assess the unilateral

intravitreal administration of OCU200 alone or in combination with an approved anti-VEGF therapy in participants with DME. This is a multicenter, open-label, dose ranging trial with three cohorts in the dose-escalation portion of the trial. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and we are actively recruiting patients.

Overview of DR and DME

DR is a sight-threatening complication of diabetes arising from the over-accumulation of glucose, which can block blood vessels in the retina and cut off blood supply, leading to the damage of blood vessels in the retina. DR is classified into two subtypes: non-proliferative DR and proliferative DR. Non-proliferative DR is the early stage of DR wherein blood vessels are unable to grow, blood vessel walls weaken, and nerve fibers in the retina may swell. Proliferative DR is the advanced stage of DR in which damaged blood vessels close off, leading to the growth of new, abnormal blood vessels in the retina. This growth of new, abnormal blood vessels in the retina can lead to scar tissue, which can result in the detachment of the retina from the back of the eye.

Complications from DR could lead to DME. In DME, bulges can protrude from the blood vessel walls, leading to the leakage of fluid and blood into the retina. This leakage results in swelling, or "edema," in the macula, which is a part of the retina. DME may occur at any stage of DR but is more likely to occur as the disease progresses. DME is the most common reason for vision loss in patients with DR.

DR and DME are the most common vision-threatening diseases in patients with diabetes. Approximately 162 million individuals are affected with DR and approximately 21 million with DME worldwide. As the population of people experiencing diabetes increases, these statistics are expected to increase, due to poor disease management, lifestyle-related changes and the aging population. There are limited treatment options available for patients with DR and DME. Current first-line treatments for DR and DME include the use of anti-vascular endothelial growth factor ("VEGF") therapy and anti-inflammatory therapy, such as corticosteroids. These treatments do not work effectively in approximately 50% of patients with DME. There is a significant need to develop a novel, differentiated therapeutic to treat DR and DME.

Additionally, current therapies target only one pathway associated with DR and DME, either angiogenesis (development of new blood vessels) with anti-VEGF therapy, such as Ranibizumab or Aflibercept, or inflammation in case of corticosteroid therapy, such as Dexamethasone or Fluocinolone. The development of a therapeutic which targets multiple causative pathways of DR and DME, such as angiogenesis, oxidation, and inflammation, would offer a potential treatment option for all patients. We believe that OCU200 possesses unique characteristics to target these pathways and has the potential to offer better treatment options for all patients with DR and DME.

Overview of Wet AMD

OCU200 also has the potential to represent a better treatment option for patients suffering from Wet AMD. Most AMD cases begin as dAMD and may progress towards the advanced "Wet" form. Wet AMD is caused by abnormal blood vessels in the retina that leak fluid or blood into the macula. The result can be irreversible damage to photoreceptor cells and rapid, severe vision loss, particularly in the center of the field of vision, causing significant functional impairment. If left untreated, neovascularization in Wet AMD patients typically results in significant vision loss and the formation of a scar under the macula. Wet AMD affects approximately 10-15% of patients with AMD but progresses more rapidly and is known to be responsible for approximately 90% of all AMD-related blindness.

AMD is a leading cause of blindness worldwide. The incidence of Wet AMD increases substantially with age, and it is expected that the number of cases of Wet AMD will increase with the growth of the elderly population. It has been estimated that approximately 20-30 million individuals worldwide suffer from Wet AMD.

Current FDA approved therapeutics for Wet AMD include intravitreal injection of either Ranibizumab or Aflibercept, which are anti-VEGF therapies. Though treatments have been effective in mitigating the disease symptoms, clinical studies suggest substantial limitations remain. For example, a significant percentage of people do not respond to therapy and experience continuous deterioration of their vision. Additionally, the repeated use of anti-VEGF therapy becomes less effective over time. Between 30-50% of people affected by Wet AMD continue to have fluid remain in the middle of the eye, also called the subretinal space, even after one to two years of treatment.

Given the above limitations of these existing treatments, we believe that a substantial unmet medical need exists for the treatment of DR, DME, and Wet AMD.

OCU200 for the Treatment of DR, DME, and Wet AMD

OCU200 is a novel fusion protein containing parts of human transferrin and tumstatin, that are already present normally in retinal tissues. Patients affected by these diseases share common symptoms, such as blurriness in vision and continued vision loss through disease progression. The formation of fragile and leaky new abnormal blood vessels leads to fluid accumulation in and around the retina, causing vision damage.

We believe OCU200 possesses unique features to efficiently target retina and choroid and inhibit vascular leakage and/or growth of new blood vessels. Tumstatin, which acts as an anti-angiogenic, anti-inflammatory, and anti-oxidative agent, is the active component of OCU200. It binds to integrin receptors, which play a crucial role in disease pathogenesis. Transferrin is expected to enhance the delivery of fused proteins across cellular barriers, including retinal barriers. OCU200 is designed to address the limitations of current therapies by targeting multiple mechanisms associated with ocular neovascularization and inflammation specifically focusing on non-responders to currently available treatment options.

A proof-of-concept study involving different animal models demonstrated the therapeutic potential of OCU200 in the treatment of DR, DME, and Wet AMD. In an animal model for DME and DR (oxygen-induced retinopathy in mice), OCU200, at a significantly lower dose (10 micrograms per eye), was comparable to existing approved anti-VEGF therapy (Eylea, 20 micrograms per eye) in preventing disease manifestation and progression. Studies in animal models for Wet AMD (laser induced choroidal neovascularization in mice and rats) suggest that OCU200 may possess comparable or slightly better activity compared to anti-VEGF control groups in preventing the formation and growth of new leaky blood vessels and subsequently disease symptoms. We believe that OCU200's distinct mechanism of action by targeting the integrin pathway could potentially provide benefit to patients, particularly to those patients that do not respond to currently approved therapies.

NEOCART (AUTOLOGOUS CHONDROCYTE-DERIVED NEOCARTILAGE) CELL THERAPY PLATFORM

We diversified our innovative pipeline in 2022 by introducing NeoCart (autologous chondrocyte-derived neocartilage), a Phase 3-ready, regenerative medicine cell therapy technology that combines breakthroughs in bioengineering and cell processing to enhance the autologous cartilage repair process. We believe NeoCart has the potential to accelerate healing and reduce pain by reconstructing a patient's previously damaged knee cartilage. In May 2022, the FDA granted an RMAT designation to NeoCart for the repair of full-thickness lesions of knee cartilage injuries in adults.

NeoCart was acquired in our reverse merger in 2019 with Histogenics Corporation ("Histogenics"). Prior to 2019, Phase 1 and Phase 2 clinical trials were conducted to demonstrate the safety and efficacy of NeoCart. These clinical trials reported a decrease in pain and improved function of the knee. Additionally, per the results of the Phase 2 clinical trial, more patients responded to NeoCart than microfracture surgery. No SAEs were reported. A Phase 3 clinical trial was conducted to demonstrate the safety and effectiveness of NeoCart as compared to microfracture surgery to treat cartilage defects in the knee. This clinical trial enrolled 249 subjects between the ages of 18 and 59. The Phase 3 clinical trial of NeoCart narrowly missed the primary endpoint of a statistically significant improvement in pain and function in a dual threshold responder analysis one year after the treatment as compared to microfracture surgery.

We have received concurrence from the FDA on the confirmatory Phase 3 clinical trial design. This study will be a randomized, controlled clinical trial designed to evaluate the efficacy and safety of NeoCart in comparison to the current standard of care, chondroplasty, in subjects with articular cartilage defects. We intend to initiate the Phase 3 trial contingent on adequate availability of funding. Our Phase 3 clinical trial will use chondroplasty as the control instead of microfracture, which was used in the Phase 3 clinical trial conducted by Histogenics. Additionally, the Phase 3 clinical trial conducted by Histogenics used a responder analysis for the co-primary endpoint (as opposed to microfracture) that included an improvement of at least 12 points in outcome compared to baseline at one year on the knee injury and OA outcome score pain assessment test and an improvement of at least 20 points in outcome compared to baseline on the International Knee Documentation Committee subjective test. In contrast, our Phase 3 clinical trial will use a co-primary efficacy endpoint defined as the mean change from baseline (as opposed to chondroplasty) to two years for the patients' Knee Injury and Osteoarthritis ("OA") Outcome Score Pain and Function (Activities of Daily Living) subscale scales. Additionally, the Phase 3 clinical trial conducted by Histogenics enrolled patients with a total lesion size of less than six cm^2, while our Phase 3 clinical trial will enroll patients with total lesion sizes between one to three cm^2.

The cartilage is a complex tissue which protects the various joints and bones in the human body. It acts as a shock absorber throughout the body withstanding significant pressure and allows for joints to glide smoothly with minimal friction. Cartilage damage can be caused by acute trauma, such as a bad fall or a sports-related injury, or by repetitive trauma, such as general wear over time. Unlike other tissues in the body, cartilage in the joints has no innate ability to repair itself, making any injury permanent. If left untreated, even a small defect can expand in size and progress to debilitating OA, ultimately requiring a joint

replacement procedure. Over 528 million individuals worldwide are diagnosed with OA. This number is expected to increase as the population of aging yet active individuals and the rates of obesity increase.

We believe the current therapies available to treat cartilage damage in the knee are suboptimal with varying outcomes due to variable cellular responses. Chondroplasty is often recommended in patients with small cartilage lesions (between one and three cm^2). This procedure is performed through small incisions on the sides of the knee with the aid of an arthroscope. During this procedure, the damaged cartilage is trimmed, and the remaining surface is smoothed. Microfracture surgery is a frequently used procedure for severe cartilage damage which yields varying outcomes from patient to patient. This surgery consists of the creation of tiny holes or "fractures" in the bone underneath the injured cartilage, leading to the formation of a blood clot in the affected area. The blood and bone marrow that form the blood clot contain stem cells, which are expected to grow into cartilage-building cells, as well as growth factors to support cell function and development of replacement cartilage matrix. Approximately 30% of patients that have undergone microfracture surgery continue to have pain and reduced knee function. Additionally, current therapies require extensive recovery time. They are often ineffective in the long term as they do not adequately address cartilage damage, which leads to additional corrective surgeries. In NeoCart, autologous culture chondrocytes on porcine collagen membrane (MACI) is used for the repair of symptomatic, single, or multiple full-thickness cartilage defects of the knee with or without bone involvement in adults less than 55 years of age. It is a three-by-five centimeter cellular sheet with a density of 500,000 cells per cm^2.

The other options for cartilage repair include osteochondral autograft transplantation ("OAT"), osteochondral allograft resurfacing ("OCA"), and autologous chondrocyte implantation ("ACI"). During OAT, damaged cartilage is removed and replaced with healthy cartilage from a non-weight-bearing area of the joint. OAT is recommended for small to medium sized lesions (between 1.5 and four cm^2) and is limited by the amount of donor tissue available, the need for open surgery, and donor site morbidity. OCA is a similar process to OAT except that the tissue is sourced from cadaveric donor bone and cartilage. OCA is recommended for large lesions (between four and 10 cm^2) and can be performed in a single procedure but is limited by the availability of cadaveric tissue. ACI is a process where cartilage cells are harvested from a non-weight bearing part of the knee and are cultured in a laboratory. They are subsequently implanted into the injured area.

Over one million arthroscopies are performed annually as a procedure to diagnose and treat issues of the joint. Patients and physicians are in need of treatment options that offer more rapid and durable recovery compared to the current treatment options. The attributes of an optimal treatment for a damaged knee cartilage involve the reduction in pain, repair of the knee cartilage, rapid return to daily activities, durable response, and a non-opioid approach. We believe NeoCart would represent a better solution to treat cartilage damage in the knee as it has the potential to solve for the limitations of the current therapies and has the potential to provide improved efficacy, long-term patient benefits, accelerated patient recovery, and predictable patient outcomes.

NeoCart is designed to treat pain at the source, improve function, and potentially prevent a patient's progression to OA. NeoCart is a three-dimensional tissue-engineered disc of new cartilage that is manufactured by growing chondrocytes, the cells responsible for maintaining cartilage health. The chondrocytes are derived from the patient on a unique scaffold. In this therapy, the patient's cells are separated from a tissue biopsy specimen and multiplied in a manufacturing facility. The cells are then infused into the scaffold, which is a three-dimensional structure that enables the proper delivery, distribution, and organization of cells in their natural environment to support tissue formation. Before NeoCart is implanted in a patient, the patient's cells and the scaffold undergo a bioengineering process in a Tissue Engineering Processor ("TEP"). The TEP is designed to mimic the conditions inside a functional joint so that the tissue is prepared to begin functioning like normal healthy cartilage prior to implantation. Once NeoCart is ready to be implanted, a bioadhesive is used to anchor NeoCart at the site of cartilage injury and seal the implant to the surrounding native cartilage. The bioadhesive is a natural, biocompatible material which acts as adhesives for biological tissue, thereby eliminating the need for complicated suturing (**Figure 10**).

Figure 10: Mechanism of the regenerative medicine cell therapy technology, NeoCart.

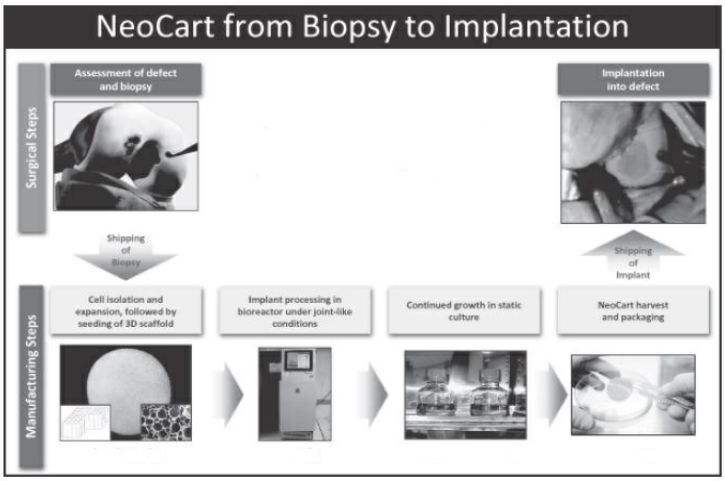


Figure 10 *demonstrates the mechanism of our regenerative medicine cell therapy technology, NeoCart. We believe NeoCart has the potential to accelerate healing and reduce pain by reconstructing a patient's previously damaged knee cartilage. In this therapy, healthy cartilage tissue is grown and implanted in the patient.*

INHALED MUCOSAL VACCINE PLATFORM

We are developing a next-generation, inhalation-based mucosal vaccine platform based on a novel ChAd vector, which includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine.

Overview of COVID-19

COVID-19, caused by the SARS-CoV-2 virus, was first reported to have surfaced in Wuhan, China in December 2019 and was declared a global pandemic by the World Health Organization ("WHO") in March 2020. COVID-19 is a highly transmissible disease that spreads from person to person through respiratory droplets that are produced when an infected person coughs, sneezes, or talks. Since being discovered, new variants of SARS-CoV-2 have emerged. New variants of a virus emerge when a mutation to the virus' genes occurs. As of November 24, 2024, SARS-CoV-2 and its variants have caused approximately over 776.9 million cases of COVID-19 and 7.1 million deaths, with the United States alone accounting for over 103 million cases and 1.2 million deaths. The JN.1 variant is a descendent lineage of SARS-CoV-2 variant BA.2.86, with the earliest sample collected on August 25, 2023 and has continued to be deemed a variant of interest by the WHO due to at least one of the following characteristics: increase in transmissibility or detrimental change in COVID-19 epidemiology, an increase in virulence or change in clinical disease presentation, or a decrease in effectiveness of public health and social measures or available diagnostics, vaccines, or therapeutics. Since being identified, JN.1 continues to be reported in multiple countries, and its prevalence has been rapidly increasing globally and now represents the vast majority of BA.2.86 descendent lineages reported. On May 11, 2023, the United States Department of Health and Human Services announced the COVID-19 public health emergency ended, yet the cases remain high as new variants emerge since the current intramuscular vaccines do not provide significant protection against respiratory mucosal infection.

Overview of the Seasonal Flu

The seasonal flu, or seasonal influenza, is an acute respiratory infection caused by influenza viruses circulating globally. In temperate climates, seasonal epidemics occur mainly during the winter, while in other regions, transmission may occur throughout the year, causing outbreaks more irregularly. The seasonal flu causes illnesses that range in severity and may lead to hospitalization and death in certain cases. The WHO estimates the seasonal flu results in three to five million cases of severe illness, and approximately 0.3 million to 0.7 million respiratory deaths annually.

The seasonal flu spreads easily and rapidly transmits in crowded areas. The seasonal flu is transmitted when an infected person coughs or sneezes and droplets containing the virus are dispersed into the air and infects those in close proximity that breathe the droplets in. The seasonal flu can also spread through physical contact, although this type of transmission is less common than airborne transmission. The flu is most commonly prevented by getting an annual flu vaccine and taking preventative actions to avoid transmission such as staying away from those who are sick, frequent handwashing, and covering coughs and sneezes. For the United States 2023 to 2024 flu season, the Centers for Disease Control and Prevention ("CDC") estimates that 40 million people were sick with influenza, 18 million people visited a healthcare provider for influenza, 470,000 had influenza-related hospitalizations, and 28,000 influenza-related deaths occurred. Vaccine manufacturers have projected that they will supply the United States with as many as 156.2 million to 170 million doses of influenza vaccines for the 2023-2024 season. Several flu antiviral drugs are also available in different dosage forms to treat the seasonal flu, including pills, liquid, an inhaled powder, or an intravenous solution. These flu antiviral drugs are only available through a prescription from a healthcare provider and are not sold over the counter.

Novel Inhaled Mucosal Vaccine Platform for the Prevention of COVID-19 and the Seasonal Flu

We are developing a next-generation, inhalation-based mucosal vaccine platform based on a novel ChAd vector, which includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine.

Our novel inhaled mucosal vaccine platform is specifically designed to generate local mucosal immunity in the nasopharyngeal region. The mucosal vaccination method has demonstrated potent induction of both mucosal and systemic immune responses, which prevents infection and spread, thereby limiting the origins of new variants. We believe our novel inhaled mucosal vaccine platform is unique as it is designed to induce mucosal immunity, which is crucial for preventing upper respiratory tract infection, as compared to intramuscular vaccines. The advantages of these inhaled mucosal vaccines include needle-free administration, the potential for increased compliance, scalable manufacturing, storage and shipping at standard refrigerated conditions, and the potential to develop multi-strain and variant-specific versions. As these vaccine candidates are being developed to be administered through inhalation, we believe our novel inhaled mucosal vaccine platform has the potential to generate rapid local immunity in the upper airways and lungs where viruses enter and infect the body, which we believe may help reduce or prevent infection and transmission as well as provide protection against new virus variants.

The Spike (S) protein of SARS-CoV-2 is the principal target for antibody-based and vaccine countermeasures. The S protein serves as the primary viral attachment and entry factor to promote SARS-CoV-2 entry into human cells. In preclinical studies that have been conducted to assess the durability, dose response, and cross-protective activity in mice, it was demonstrated that a single dose of our inhaled mucosal COVID-19 vaccine induced durably high neutralizing and antibody effector responses in serum and S protein specific IgG and Immunoglobulin A, which is essential for reducing infection and transmission of COVID-19. This approach represents a potential universal booster, regardless of previous COVID-19 vaccination. OCU520, our combination quadrivalent seasonal flu and COVID-19 vaccine, is designed to provide the unique ease of getting both an annual COVID-19 booster vaccine and an annual seasonal flu vaccine in one vaccine.

Pursuant to the WU License Agreement, we obtained the rights to develop, manufacture, and commercialize a mucosal COVID-19 vaccine in the Mucosal Vaccine Territory. In addition, we internally developed technology related to the flu and COVID-19's vaccine design and filed intellectual property. In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. OCU500 will be tested via two different mucosal routes, inhalation and intranasal delivery. NIAID intends to initiate a Phase 1 clinical trial in the second quarter of 2025. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

COMPETITION

The biotechnology industry is characterized by rapidly advancing technologies as well as a strong emphasis on intellectual property leading to a highly competitive environment for the development and commercialization of therapeutic products, regenerative medicines, and vaccines. We face competition with respect to our current product candidates and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future. We face competition from many different sources, including from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization. We plan to compete in the segments of pharmaceutical, biotechnological, and other related markets with therapeutics, regenerative medicines, and vaccines that have an acceptable safety profile and target commercially attractive indications.

The development and commercialization of gene therapies is highly competitive. We are aware of several companies focusing on gene therapies for various ophthalmic indications including Applied Genetic Technologies Corporation, as acquired by Syncona Limited, Astellas Pharma Inc., MeiraGTx Holdings plc in partnership with Janssen Pharmaceuticals, Inc., Nanoscope Therapeutics Inc., REGENXBIO Inc., Novartis AG, F. Hoffmann-La Roche AG ("Roche AG"), Kiora Pharmaceuticals, Inc., Genentech, Inc. in partnership with Lineage Cell Therapeutics, Inc., and Luxturna, the product developed by Spark Therapeutics, Inc. and marketed by Roche AG, is currently the only gene therapy approved to treat IRDs in the United States which addresses only mutations in the one gene, *RPE65*. The mutation associated with the *RPE65* gene represents just one of more than 125 mutated genes linked to RP and LCA.

The regenerative medicine sector is characterized by innovative science, rapidly advancing technologies, and a strong emphasis on proprietary products. The competitive landscape in the field of articular cartilage repair in the United States is emerging and has stimulated a substantial amount of interest from companies developing tissue repair solutions. Companies that may compete with our NeoCart product candidate include Vericel Corporation's MACI, the only FDA-approved ACI product in the United States, and Aesculap Biologics, LLC's NOVOCART 3D, which is currently enrolling subjects in their Phase 3 clinical trial.

We face, and will continue to face, intense competition from companies as well as institutions that are pursuing or have commercialized vaccines that would compete with our novel inhaled mucosal vaccine platform, if commercialized. The competitive landscape of COVID-19 vaccines has been rapidly developing since the beginning of the COVID-19 pandemic and includes competitors such as Pfizer Inc./BioNTech SE, Moderna, Inc., AstraZeneca PLC, Novavax, Inc., Sinovac Biotech Ltd., Gamaleya Research Institute of Epidemiology and Microbiology, and Center for Genetic Engineering and Biotechnology. Each of the aforementioned vaccines have been authorized or approved in at least one country within the Ocugen Mucosal Vaccine Territory and are intramuscular vaccines. CanSinoBIO's Convidecia Air, an intranasal vaccine targeting COVID-19, has been approved in China. Other competitors for our novel inhaled mucosal vaccine platform include CyanVac LLC, Meissa Vaccines, Inc., Codagenix, Inc., Intravacc B.V., McMaster University, and Tetherex Pharmaceuticals Corporation. Companies such as Pfizer Inc./BioNTech SE, Moderna, Inc., CureVac N.V in partnership with GSK plc, Vivaldi Biosciences Inc., and Novavax, Inc. are also in the process of developing a combination vaccine that will protect against COVID-19 and the seasonal flu. Vivaldi Biosciences Inc. is also currently undergoing clinical trials for their intranasal vaccine for the seasonal flu.

The development and commercialization of biologic products is highly competitive as well. Companies that may compete with our OCU200 product candidate include Roche AG, Regeneron Pharmaceuticals, Inc., AsclepiX Therapeutics, Inc., Outlook Therapeutics, Inc., Novartis AG, Oxurion NV, Unity Biotechnology, Inc., Opthea Limited, and 4D Molecular Therapeutics, Inc. Roche AG, Regeneron Pharmaceuticals, Inc., and Novartis AG have marketed anti-VEGF products.

Many of our competitors, either alone or with strategic partners, may have significantly greater financial resources to support research and development, manufacturing, preclinical studies, and clinical trials, as well as regulatory, commercialization, and marketing efforts. These organizations also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites, patient registration for clinical trials, licensing or acquiring technologies necessary for our programs, and in our commercialization efforts if our product candidates are approved. Early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors.

MANUFACTURING AND SUPPLY

We utilize our in-house expertise and know-how as well as the expertise and know-how of our industry leading manufacturing partners to develop and scale up our manufacturing processes for both the clinical and commercial supply of our product candidates. Our in-house expertise includes personnel with extensive product development and commercialization experience who actively manage our manufacturing partners that produce products in our product candidate pipeline. Our current manufacturing partners have state-of-the-art facilities with significant expertise in biotechnology manufacturing.

Clinical Supply of Our Modifier Gene Therapy Platform

We have a co-development and commercialization agreement with CanSinoBIO with respect to the development and commercialization of our modifier gene therapy platform including OCU400, OCU410 and OCU410ST. CanSinoBIO is responsible for the CMC development and manufacture of clinical supplies of such product candidates and is responsible for the costs associated with such activities. CanSinoBIO has an exclusive license to develop, manufacture, and commercialize our modifier gene therapy platform in and for China, Hong Kong, Macau, and Taiwan (the "CanSinoBIO Territory"), and we maintain exclusive development, manufacturing, and commercialization rights with respect to our modifier gene therapy platform outside the CanSinoBIO Territory (the "Company Territory").

We partner with CanSinoBIO for the process development, manufacturing, testing, and release of drug product candidates for use in IND-enabling studies and clinical trials. We perform discovery and analytical development activities in our research and development lab. The partnership with CanSinoBIO enables us in completing manufacturing, with release of clinical trial materials in an expedited manner and helps in mitigating the risk of delay that can be associated when working with highly competitive CDMOs that have long wait times with regard to gene therapy manufacturing. Although we rely on our partnership for manufacturing, we have personnel with extensive experience in gene therapy manufacturing to oversee and guide the process and analytical development, scale-up, release, and stability testing at our partner site. We perform periodic audits of our manufacturing partner to confirm compliance with applicable regulations.

For more information about our partnership with CanSinoBIO, see "—License and Development Agreements—Co-Development and Commercialization Agreement with CanSinoBIO" and see Note 3 in our notes to the consolidated financial statements included elsewhere in this Annual Report.

Clinical and Commercial Supply of NeoCart

We have completed renovating an existing facility into a current GMP facility in accordance with the FDA's regulations in support of NeoCart manufacturing for Phase 3 clinical trial material.

Clinical Supply of Inhaled Mucosal Vaccine Platform

In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. Project NextGen is a $5 billion multi-government agency initiative to develop the next generation of vaccines and therapeutics to combat the spread of COVID-19. NIAID, with funding from Project NextGen, will cover the full cost of the clinical trials, including operations and related analysis. We will be responsible for providing clinical trial materials and upon completion will have full right of reference to the findings, which we believe will provide clinical evidence to support the further development of our lead mucosal vaccine candidate.

Clinical Supply of OCU200

In October 2020, we entered into a manufacturing agreement with a CDMO for the manufacture of OCU200. Under the manufacturing agreement, our CDMO will manage all CMC and clinical manufacturing activities for OCU200. We have completed the technology transfer of manufacturing processes to our CDMO and have produced clinical trial materials to initiate the planned Phase 1 clinical trial. In April 2023, the FDA placed our IND application to initiate a Phase 1 trial targeting DME on clinical hold, as part of the FDA's request for additional information related to CMC. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and we are actively recruiting patients.

LICENSE AND DEVELOPMENT AGREEMENTS

We are party to license and development agreements under which we license or co-own patents, patent applications, technical information, and other intellectual property for our product candidates. Certain diligence and financial obligations are tied to these agreements. We consider the following agreements to be material to our business.

Modifier Gene Therapy Program

Exclusive License Agreement with SERI

In December 2017, we entered into an exclusive license agreement with SERI, which was amended in January 2021 (as amended, the "SERI Agreement"). The SERI Agreement gives us an exclusive, worldwide, sublicensable license to patent rights, biological materials, and technical information for NHR genes *NR1D1*, NR2E3 (OCU400), *RORA* (OCU410 and OCU410ST), Nuclear Protein 1, Transcriptional Regulator ("*NUPR1*"), and Nuclear Receptor Subfamily 2 Group C Member 1 ("*NR2C1")*. The January 2021 amendment to the SERI Agreement additionally granted us rights in co-owned intellectual property pursuant to certain patent applications and provisional patent applications at the time of the amendment. Under the SERI Agreement, we may make, have made, use, offer to sell, and import licensed products, and must use commercially reasonable efforts to bring one or more licensed products to market as soon as reasonably practicable.

SERI maintains control of patent preparation, filing, prosecution, and maintenance. We are responsible for SERI's out-of-pocket expenses related to the filing, prosecution, and maintenance of the licensed patent rights. In the event that SERI decides to discontinue the prosecution or maintenance of the licensed patent rights, we have the right, but not the obligation, to file for,

or continue to prosecute, maintain, or enforce such licensed patent rights. We have assumed prosecution of certain licensed patent rights under the SERI Agreement.

Co-Development and Commercialization Agreement with CanSinoBIO

We entered into the CanSinoBIO Agreement with CanSinoBIO with respect to the development and commercialization of our modifier gene therapy product candidates, OCU400, OCU410, and OCU410ST. The CanSinoBIO Agreement was originally entered into in September 2019 with regards to OCU400 and was subsequently amended in September 2021 and November 2022 to include OCU410 and OCU410ST, respectively. Pursuant to the CanSinoBIO Agreement, we are collaborating with CanSinoBIO on the development of our modifier gene therapy platform. CanSinoBIO is responsible for the CMC development and manufacture of clinical supplies of such product candidates and is responsible for the costs associated with such activities. CanSinoBIO has an exclusive license to develop, manufacture, and commercialize our modifier gene therapy platform in and for the CanSinoBIO Territory, and we maintain exclusive development, manufacturing, and commercialization rights with respect to our modifier gene therapy platform in the Company Territory.

CanSinoBIO will pay us an annual royalty between mid- and high-single digits based on Net Sales (as defined in the CanSinoBIO Agreement) of the products included in our modifier gene therapy platform in the CanSinoBIO Territory. We will pay CanSinoBIO an annual royalty between low- and mid-single digits based on Net Sales of the products included in our modifier gene therapy platform in the Company Territory.

NeoCart

License Agreement with Purpose

In December 2005, Histogenics entered into an exclusive agreement (the "Purpose Agreement") to sublicense certain technology from Purpose, which we assumed as a result of our reverse merger with Histogenics. Purpose entered into the original license agreement ("BWH-Purpose Agreement") with Brigham and Women's Hospital, Inc. ("BWH") in August 2001. The BWH-Purpose Agreement granted Purpose an exclusive, royalty-bearing, worldwide, sublicensable license, under its rights in licensed patents and patent applications co-owned by BWH and Purpose to make, use, and sell (1) an apparatus for cultivating a cell or tissue, (2) cell or tissue products made using such apparatus, (3) cell or tissue products made using processes for cultivating a cell or tissue as disclosed in the licensed patents and patent applications, and (4) any apparatus that cultivates cells or tissues using such processes, in each case, whose manufacture, use, or sale is covered by a valid claim of the licensed patents and patent applications, only for therapeutic use.

The Purpose Agreement was amended and restated in June 2012, pursuant to which Purpose granted Histogenics outside of Japan: (a) exclusive rights to all of Purpose's technology (owned or licensed) related to the exogenous tissue processors, which is used in the development of NeoCart, (b) continued supply of exogenous tissue processors, and (c) rights to manufacture the exogenous tissue processors at any location we choose. In exchange for such consideration, Purpose was granted an exclusive license in Japan for the use of all of our NeoCart technology and was reimbursed for development costs on a multi-unit exogenous tissue processor. In May 2016, the Purpose Agreement was amended, whereby Histogenics reacquired the development and commercialization rights to NeoCart in Japan.

The Purpose Agreement, as amended, provides us with the ability, worldwide, to (i) use, make, have made, sell, offer for sale, import or otherwise exploit products or services covered by claims of Purpose's patents and (ii) use, reproduce, modify, create derivative works of and otherwise exploit Purpose's technology for the design, development, manufacture, testing, support, and commercialization of any product or service that incorporates or builds upon Purpose's technology, in each case, only in connection with articular cartilage, ligaments, tendons, and meniscus. Purpose retains the right to sell its single unit exogenous tissue processer machines to research institutes for general but noncommercial use anywhere in the world.

Vaccines

Exclusive License Agreement with Washington University

In September 2022, we entered into the WU License Agreement with Washington University, pursuant to which we were granted an exclusive, sublicensable, royalty-bearing license to patent rights for a mucosal COVID-19 vaccine, as well as a license to certain tangible research property and technical information necessary to exploit the patent rights within the United States, Europe, and Japan. In January 2023, we amended the WU License Agreement to add the countries of South Korea, Australia, and China to the Mucosal Vaccine Territory, and in November 2023, we further amended the WU License Agreement to add Hong Kong to the Mucosal Vaccine Territory.

Pursuant to the WU License Agreement, we may make, have made, sell, offer for sale, use, market, promote, distribute, export, and import licensed products in the Mucosal Vaccine Territory. We will use commercially reasonable efforts to develop, manufacture, promote, and sell the licensed products in the Mucosal Vaccine Territory.

Washington University maintains control of patent preparation, filing, prosecution, and maintenance. We are responsible for Washington University's out-of-pocket expenses related to the preparation, filing, prosecution, issuance, and maintenance of the licensed patent rights incurred pursuant to the WU License Agreement.

NIAID Project NextGen Clinical trial support

In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. Project NextGen is a $5 billion multi-government agency initiative to develop the next generation of vaccines and therapeutics to combat the spread of COVID-19. NIAID, with funding from Project NextGen, will cover the full cost of the clinical trials, including operations and related analysis. We will be responsible for providing clinical trial materials and upon completion will have full right of reference to the findings, which we believe will provide clinical evidence to support the further development of our lead mucosal vaccine candidate.

Novel Biologic Therapy for Retinal Diseases

Exclusive License Agreement with the University of Colorado

In March 2014, we entered into an exclusive license agreement with CU, which was amended in January 2017 and clarified by a letter of understanding in November 2017 (as amended and clarified, the "CU Agreement"). The CU Agreement gives us an exclusive, worldwide, sublicensable license to patents for OCU200 to make, have made, use, import, offer to sell, sell, have sold, and practice the licensed products in all therapeutic applications. Under the CU Agreement, we must use commercially reasonable efforts to develop, manufacture, sublicense, market, and sell the licensed products and have assumed primary responsibility for preparing, filing, and prosecuting broad patent claims for OCU200 for CU's benefit. Further, we have assumed primary responsibility for all patent activities, including all costs associated with the perfection and maintenance of the patents for OCU200.

INTELLECTUAL PROPERTY

Our success depends in part upon our ability to protect our core technologies and intellectual products. We have applied, obtained, and licensed patent protection for our product candidates. We intend to maintain and defend our intellectual property rights to protect our technology, inventions, processes, and improvements that are commercially important to the development of our business. There is no guarantee that any of our current or future intellectual property will advance the commercial success of our product candidates. There is also no guarantee patents will be issued or registered for any pending patent applications or patent applications that we may file in the future. Our commercial success also depends in part on our non-infringement of the patents and proprietary rights of third parties.

As of February 24, 2025, our patent portfolio for our product candidates included a total of 18 issued patents in the United States, 45 issued or registered patents in foreign countries, 10 pending patent applications in the United States, and 20 pending patent applications in foreign countries. Our issued or registered patents and pending patent applications include those licensed from SERI, Purpose, Washington University, and CU. Certain issued patents and pending patent applications cover multiple of our product candidates. Our intellectual property includes compositions of matter, methods of use, product candidates, and other proprietary technology. As of February 24, 2025, we had exclusive rights or owned rights to: (i) two issued United States patents, one pending United States patent applications, and one pending foreign patent applications related to OCU400; (ii) one pending United States patent applications, and three pending foreign patent applications related to OCU410 and OCU410ST; (iii) 15 issued United States patents; six pending United States patent applications, 20 issued or registered foreign patents, and nine pending foreign patent applications related to NeoCart; (iv) two pending United States patent applications and seven pending foreign patent applications related to OCU500, OCU510 and OCU520; and (v) one issued United States patent and 25 issued or registered foreign patents related to OCU200. Our current portfolio of issued patents in the United States and issued or registered patents in foreign countries related to our product candidates expire between 2025 and 2038.

In some instances, we may need to license additional patents and trade secrets to commercialize our product candidates in certain territories. In addition to patents, we may rely, in some circumstances, on trade secrets to protect our technology. We seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, by confidentiality and invention assignment agreements with our employees, consultants, scientific advisors, and contractors. We

also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems.

GOVERNMENT REGULATION AND PRODUCT APPROVAL

Government authorities in the United States, at the federal, state, and local level, and in other countries including Canada, extensively regulate, among other things, the research, development, testing, approval, manufacture, packaging, storage, recordkeeping, monitoring and reporting, labeling, advertising, promotion, distribution, marketing, sales, import, and export of biotechnological and drug products such as those we are developing. In addition, labelers of marketed biotechnology and drug products (the entity owning the National Drug Code listed for a marketed product) participating in Medicaid and Medicare are required to comply with mandatory price reporting, discounts, rebates, and other requirements. The processes for obtaining regulatory approvals in the United States and in other countries including Canada, along with compliance with applicable statutes and regulations, require the expenditure of substantial time and financial resources.

From time to time, legislation is drafted, introduced, passed in Congress and signed into law that could significantly change the statutory provisions governing the approval, manufacturing, and marketing of products regulated by the FDA. In addition to new legislation, FDA regulations, guidances, and policies are often revised or reinterpreted by the agency in ways that may significantly affect the manner in which pharmaceutical products are regulated and marketed.

FDA Regulation

In the United States, the FDA regulates biologics and drug products under the Federal Food, Drug, and Cosmetic Act ("FDCA") and its implementing regulations. In addition, biological products are regulated under the Public Health Service Act ("PHSA") and its implementing regulations. The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies, and formulation studies in compliance with the FDA's Good Laboratory Practice ("GLP") regulations, applicable requirements for the human use of laboratory animals, such as the Animal Welfare Act ("AWA"), or other applicable regulations;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin at United States clinical trial sites;
- approval by an Institutional Review Board ("IRB") for each clinical site, or centrally, before a clinical trial may be initiated at that site;
- adequate and well-controlled human clinical trials to establish the safety and efficacy, in the case of a drug product candidate, or safety, purity, and potency, in the case of a biological product candidate, for its intended use, performed in accordance with Good Clinical Practice ("GCP") and additional requirements for the protection of human research subjects and their health information;
- development of manufacturing processes to ensure the product candidate's identity, strength, quality, purity, and potency in compliance with current GMP;
- submission to the FDA of a New Drug Application ("NDA"), in the case of a drug product candidate, or a BLA, in the case of a biological product candidate, including results of preclinical testing, detailed information about the CMC, and proposed labeling and packaging for the product candidate;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the products are produced to assess compliance with current GMP, and to assure that the facilities, methods, and controls are adequate to preserve the therapeutics' identity, strength, quality, purity, and potency as well as satisfactory completion of an FDA inspection of selected clinical sites, selected clinical investigators to determine GCP compliance, and payment of user fees; and
- FDA review and approval of the NDA, or licensure of a BLA, to permit commercial marketing for particular indications for use, including agreement on post-marketing commitments, if applicable.

Preclinical Studies and IND Submission

The testing and approval process of product candidates requires substantial time, effort, and financial resources. Satisfaction of FDA pre-market approval requirements typically takes many years, and the actual time required may vary substantially based

upon the type, complexity, and novelty of the product or disease. Preclinical studies include laboratory evaluation of chemistry, pharmacology, toxicity, and product formulation, as well as animal studies to assess potential safety and efficacy. Such studies must generally be conducted in accordance with GLP, the AWA, and other applicable regulations and requirements. Prior to commencing the first clinical trial at a United States investigational site with a product candidate, an IND sponsor must submit the results of the preclinical tests and preclinical literature, together with manufacturing information, analytical data, any available clinical data or literature, and proposed clinical study protocols, among other things, to the FDA as part of an IND submission. Some preclinical studies may continue even after the IND is in effect.

An IND application becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, notifies the applicant of safety concerns or questions related to one or more proposed clinical trials and places the trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial may begin. A separate submission to an existing IND application must also be made for each successive clinical trial conducted during product development.

Clinical Trials

Clinical trials involve the administration of the investigational product to human subjects (healthy volunteers or patients) under the supervision of qualified investigators. Clinical trials must be conducted in accordance with federal regulations and GCP requirements, which include the requirements that all research subjects provide their informed consent in writing for their participation in any clinical trial, as well as the review and approval of the study by an IRB. 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 trial, the trial procedures, the parameters to be used in monitoring safety, the effectiveness criteria to be evaluated, and a statistical analysis plan. A protocol for each clinical trial, and any subsequent protocol amendments, must be submitted to the FDA as part of the IND submission. If a product candidate is being investigated for multiple intended indications, separate IND applications may also be required. In addition, an IRB at each study site participating in the clinical trial and/or a central IRB must review and approve the plan for any clinical trial, informed consent forms, and communications to study subjects before a study commences at that site. An IRB is charged with protecting the welfare and rights of trial participants, and considers, among other things, whether the risks to individuals participating in the trials are minimized and are reasonable in relation to anticipated benefits, and whether the planned human subject protections are adequate. The IRB must continue to oversee the clinical trial while it is being conducted. Progress reports detailing the results of the clinical trials must also be submitted at least annually to the FDA and the IRB and more frequently if SAEs or other significant safety information is found.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Sponsors are required to submit periodic progress reports and safety reports to FDA throughout the clinical development program. Phase 1, Phase 2, and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. If the FDA issues a clinical hold halting a clinical trial, the agency must notify the IND sponsor of the grounds for the hold. Any identified deficiencies must be resolved before the FDA will lift the hold and allow the clinical trial to begin or resume. There is no guarantee the FDA will ever lift a clinical hold once put in place. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements or if the trial poses an unexpected serious harm to subjects. The FDA or an IRB may also impose conditions on the conduct of a clinical trial. Clinical trial sponsors may also choose to discontinue clinical trials as a result of risks to subjects, a lack of favorable results, or changing business priorities.

The manufacture of investigational drugs and biologics for the conduct of human clinical trials is subject to current GMP requirements. Investigational drugs, biologics, active ingredients, 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 the United States export requirements under the FDCA.

In general, for purposes of NDA and BLA approval, human clinical trials are typically conducted in three sequential phases, which may overlap or be combined.

- *Phase 1* — Studies are initially conducted in a small group of healthy human volunteers or subjects (e.g., 10 to 20 subjects) to test the product candidate for safety, dosage tolerance, structure-activity relationships, mechanism of action, absorption, metabolism, distribution, and excretion. If possible, Phase 1 trials may also be used to gain an initial indication of product effectiveness.

- *Phase 2* — Controlled studies are conducted in larger but still limited subject populations with a specified disease or condition to evaluate preliminary efficacy, identify optimal dosages, dosage tolerance and schedule, possible adverse effects and safety risks, and expanded evidence of safety.
- *Phase 3* — These adequate and well-controlled clinical trials are undertaken in expanded subject populations (e.g., several hundred to several thousand patients), generally at geographically dispersed clinical trial sites, to generate enough data to provide statistically significant evidence of clinical efficacy and safety of the product candidate for approval, to establish the overall risk-benefit profile of the product candidate, and to provide adequate information for the labeling of the product candidate. Typically, two Phase 3 trials are required by the FDA for product approval. Under some limited circumstances, however, the FDA may approve an NDA or BLA based upon a single Phase 3 clinical study.

The FDA may also require, or companies may conduct, additional clinical trials for the same indication after a product is approved. These are referred to as Phase 4 studies and may be made a condition to be satisfied after approval. The results of Phase 4 studies can confirm or refute the effectiveness of a product candidate and can provide important long-term safety information.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with current GMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, manufacturers must develop methods for testing the identity, strength, quality, potency, and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Marketing Application Submission, Review by the FDA, and Marketing Approval

Assuming successful completion of the required clinical and preclinical testing, the results of product development, including CMC, non-clinical studies, and clinical trial results, including negative or ambiguous results, as well as positive findings, are all submitted to the FDA, along with the proposed labeling, as part of an NDA, in the case of a drug, or BLA, in the case of a biologic, requesting approval to market the product for one or more indications. In most cases, the submission of a marketing application is subject to a substantial application user fee.

In addition, under the Pediatric Research Equity Act ("PREA"), a BLA or NDA or supplement to a BLA or NDA for a new active ingredient, indication, dosage form, dosage regimen, or route of administration, must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after the approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Orphan products are also exempt from the PREA requirements.

The FDA also may require submission of a risk evaluation and mitigation strategy ("REMS") to ensure that the benefits of the product candidate outweigh the risks. Following product approval, a REMS may also be required by the FDA if new safety information is discovered and the FDA determines that a REMS is necessary to ensure that the benefits of the product continue to outweigh the risks. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription, or dispensing of products.

Once the FDA receives an application, it generally takes 60 days to review the NDA or BLA (collectively, the "marketing application") to determine if it is substantially complete to permit a substantive review, before it accepts the application for filing. The FDA may refuse to review any application that it deems incomplete or not properly reviewable at the time of submission and may request additional information. If the submission is accepted for filing, the FDA begins an in-depth review of the marketing application. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act ("PDUFA"), the FDA aims to complete its initial review of a marketing application and respond to the applicant within 10 months from the filing date for a standard marketing application, and within six months from the filing date for a priority marketing application. The FDA does not always meet its PDUFA goal dates for standard and priority marketing applications, and the review process is often significantly extended by FDA requests for additional information or clarification.

The FDA may also refer certain applications to an advisory committee. Before approving a product candidate for which no active ingredient (including any ester or salt of an active ingredient) has previously been approved by the FDA, the FDA must either refer that product candidate to an external advisory committee or provide in an action letter a summary of the reasons

why the FDA did not refer the product candidate to an advisory committee. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA reviews applications to determine, among other things, whether a product candidate meets the agency's approval standards and whether the manufacturing methods and controls are adequate to assure and preserve the product's identity, strength, quality, potency, and purity. Before approving a marketing application, the FDA typically will inspect the facility or facilities where the product is manufactured, referred to as a Pre-Approval Inspection. The FDA will not approve an application unless it determines that the manufacturing processes and facilities, including contract manufacturers and subcontractors, are in compliance with current GMP requirements and are adequate to assure consistent production of the product within required specifications. Additionally, before approving a marketing application, the FDA will inspect one or more clinical trial sites to assure compliance with GCP. To assure current GMP and GCP compliance, an applicant will incur significant expenditure of time, money, and effort in the areas of training, recordkeeping, production, and quality control.

After evaluating the marketing application and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter ("CRL"). A CRL indicates that the review cycle for the application is complete and the application is not ready for approval. It also describes all of the specific deficiencies that the FDA identified. A CRL generally contains a statement of specific conditions that must be met in order to secure final approval of the marketing application and may require additional clinical or preclinical testing in order 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.

Even if the FDA approves a product, it may limit the approved indications or populations for use of the product, require that contraindications, warnings, or precautions be included in the product labeling, including a boxed warning, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a product's safety and efficacy after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product.

Pediatric Exclusivity

Pediatric exclusivity is one type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity and patent periods. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric studies, and the applicant agreeing to perform and report on the requested studies within the statutory timeframe.

Orphan Products

The Orphan Drug Act provides incentives for the development of products for rare diseases or conditions. Specifically, sponsors may apply for and receive ODD if a product candidate is intended to treat rare diseases or conditions, which generally are diseases or conditions affecting less than 0.2 million individuals in the United States, or affecting more than 0.2 million individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States will be recovered from the United States sales. ODD must be requested before submitting an NDA or BLA. Additionally, sponsors must present a plausible hypothesis for clinical superiority to obtain ODD if there is a product already approved by the FDA that is considered by the FDA to be the same and is intended for the same indication. This hypothesis must be demonstrated to obtain ODD exclusivity. If granted, ODD entitles a party to certain pre-approval financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and certain user-fee waivers. After the FDA grants ODD, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. ODD does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA or BLA applicant to receive FDA approval for a particular active moiety to treat a particular disease with ODD generally is entitled to a seven-year exclusive marketing period in the United States for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety, or providing a major contribution to patient care. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

Patent Term Restoration and Regulatory Data Exclusivity

In the United States and elsewhere, certain regulatory exclusivities and patent rights can provide an approved drug product with protection from certain competitors' products for a period of time and within a certain scope. If approved, drug and biologic products may also be eligible for periods of the United States patent term restoration. If granted, patent term restoration extends the patent life of a single unexpired patent, that has not previously been extended, for a maximum of five years. The total patent life of the product with the extension also cannot exceed 14 years from the product's approval date. Subject to prior limitations, the period of extension is calculated by adding half of the time from the effective date of an IND application to the initial submission of a marketing application, and all of the time between the submission of the marketing application and its approval. This period may also be reduced by any time that the applicant did not act with due diligence. Only one patent claiming each approved product is eligible for restoration and the patent holder must apply for restoration within 60 days of approval. The United States Patent and Trademark Office ("USPTO"), in consultation with the FDA, reviews and approves the application for patent term restoration.

Additionally, under the PHSA, innovator biological products, or reference products, are entitled to 12 years of exclusivity. The FDA must wait four years after licensure of a biologic product under a BLA before accepting a filing for a biosimilar version of the reference product, and the FDA cannot approve a biosimilar version of the reference product until 12 years after the reference product was approved under a BLA.

Special FDA Expedited Review and Approval Programs

The FDA has various programs that are intended to expedite or simplify the process for the development and FDA review of certain product candidates that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs or present a significant improvement over existing therapy. The purpose of these programs is to provide important new therapeutics to patients earlier than under standard FDA review procedures. These expedited programs include fast track designation, breakthrough therapy designation, priority review, accelerated approval, and RMAT designation. Each of these programs has its own features and qualifying criteria. A sponsor must submit a request for fast track designation, breakthrough therapy designation, or priority review, which may or may not be granted by the FDA.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product candidate is intended to treat a serious or life threatening disease or condition and demonstrates the potential to address an unmet medical need. If fast track designation is obtained, sponsors may be eligible for more frequent development meetings and correspondence with the FDA. In addition, the FDA may initiate review of sections of an application before the application is complete. This "rolling review" is available if the applicant provides and the FDA approves a schedule for the remaining information.

To be eligible for breakthrough therapy designation, the FDA must determine, based on the request of the sponsor, that a product candidate is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Products designated as breakthrough therapies are eligible for intensive guidance on an efficient development program beginning as early as Phase 1 trials, a commitment from the FDA to involve senior managers and experienced review staff in a proactive collaborative and cross-disciplinary review, rolling review, and the facilitation of cross-disciplinary review.

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising regenerative medicine products, including genetic therapies. A regenerative medicine advanced therapy is eligible for RMAT designation if it is intended to treat, modify, reverse, or cure a serious or life threatening disease or condition, and preliminary clinical evidence indicates that the drug or therapy has the potential to address unmet medical needs for such disease or condition. Similar to breakthrough therapy designation, RMAT designation provides the benefits of intensive FDA guidance on efficient drug development, including the ability for early interactions with FDA to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of a BLA, and other opportunities to expedite development and review.

The FDA may give a priority review designation to product candidates that are intended to treat serious conditions and, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of the serious condition. A priority review means that the goal for the FDA is to review an application within six months from the filing date, rather than the standard review of 10 months under current PDUFA guidelines.

Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be developed and approved under the accelerated approval pathway, which means the FDA may approve the product candidate based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. A drug or biologic candidate approved under the accelerated approval pathway is generally subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect of the product. Under the Food and Drug Omnibus Reform Act of 2022 ("FDORA"), the FDA is now permitted to require, as appropriate, that confirmatory trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Further, under FDORA, the FDA has increased authority for expedited procedures to withdraw the accelerated approval of a drug or biologic if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product.

Even if a drug or biological product qualifies for one or more of these programs, the FDA may later decide that such product no longer meets the qualification criteria, and therefore, that the sponsor is no longer eligible for the program benefits, including the benefit of a shortened time period for FDA review or approval. Additionally, fast track designation, priority review, accelerated approval, breakthrough therapy designation, and RMAT designation do not change the standards for approval and do not necessarily shorten the overall time it takes a sponsor to obtain FDA approval or the costs of obtaining it.

Post-Approval Requirements

Any products manufactured or distributed pursuant to FDA approvals are subject to extensive and continuing regulation by the FDA, including, among other things, requirements related to manufacturing, recordkeeping, and reporting, including adverse experience reporting, deviation reporting, shortage reporting, and periodic reporting, product sampling and distribution, advertising, marketing, promotion, certain electronic records and signatures, and post-approval obligations imposed as a condition of approval, such as Phase 4 clinical trials, REMS, and surveillance to assess safety and effectiveness after commercialization.

Future FDA inspections may identify current GMP compliance issues at manufacturing facilities or at the facilities of third-party suppliers that may disrupt production or distribution or require substantial resources to correct and prevent recurrence of any deficiencies and could result in fines or penalties by regulatory authorities.

After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to FDA review and approval. 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 required to register their establishments with the FDA and certain state agencies, list their products, and are subject to periodic announced and unannounced inspections by the FDA and these state agencies for compliance with current GMP and other requirements. Regulatory authorities may undertake regulatory enforcement action, withdraw product approvals, require label modifications, or request product recalls, among other actions, if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Changes to the manufacturing process are strictly regulated and often require FDA approval or notification before being implemented. FDA regulations also require investigation and correction of any deviations from current GMP and specifications, and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use.

The FDA also strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. 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. Drug and biological product 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 ("FCA"), 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, newly discovered or developed safety or efficacy data may require changes to a product's approved labeling, including the addition of new warnings and contraindications.

In addition, the distribution of prescription drug and biological products 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 drug and biological product samples and impose requirements to ensure accountability in distribution. Free trial or starter prescriptions provided through pharmacies are also subject to regulations under the Medicaid Drug Rebate Program ("MDRP") and potential liability under anti-kickback and false claims laws.

Moreover, the Drug Supply Chain Security Act imposes obligations on sponsors of drug and biological 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.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in significant regulatory actions. Such actions may include refusal to approve pending applications, license or approval suspension or revocation, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, Form FDA 483s, cyber letters, modification of promotional materials or labeling, provision of corrective information, imposition of post-market requirements including the need for additional testing, imposition of distribution or other restrictions under a REMS, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, FDA debarment, injunctions, fines, consent decrees, corporate integrity agreements, suspension and debarment from government contracts, refusal of orders under existing government contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement, civil or criminal penalties including fines and imprisonment, and adverse publicity, among other adverse consequences.

Additional controls for biologics

To help reduce the increased risk of the introduction of adventitious agents through biological products, 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 may also be subject to official lot release as a condition of approval. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits to FDA a release protocol showing the results of all of the manufacturer's tests performed on the lot, and if required, samples of each lot of the product.

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 during product development that 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 potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a prolonged period of time.

Fraud and Abuse, Data Privacy and Security, and Transparency Laws and Regulations

Our business activities, including but not limited to, research, marketing, sales, promotion, distribution, medical education, and other activities following product approval are subject to laws and regulations by numerous federal and state regulatory and law enforcement authorities in the United States in addition to the FDA, including potentially the Department of Justice, the Department of Health and Human Services and its various divisions, including the Centers for Medicare and Medicaid Services ("CMS") and the Health Resources and Services Administration, the Department of Veterans Affairs, the Department of Defense, and state and local governments. Our business activities must comply with numerous healthcare laws, including but not limited to, the federal civil FCA, the federal Anti-Kickback statue, and other laws and regulations as well as data privacy and security laws and regulations, which are described below, as well as state and federal consumer protection and unfair competition laws. Moreover, to the extent that we license the right to sell our product, if approved, to another entity under that entity's labeler code, the licensee would have regulatory responsibilities, including healthcare, reimbursement, pricing, and reporting regulatory responsibilities.

The federal Anti-Kickback Statute, prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting, or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering, or arranging for or recommending the purchase, lease, or order, or the referral to another for the furnishing or arranging of any item or service reimbursable under Medicare, Medicaid, or other federal healthcare programs, in whole or in part. The term "remuneration" has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between biotechnological industry members on one hand and prescribers, purchasers, formulary managers, and beneficiaries on the other. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of a federal healthcare covered business, including purchases of products paid by federal healthcare programs, the statute has been violated. A violation of the Anti-Kickback Statute may be established without proving actual knowledge of the statute or specific intent to violate it. The government or a whistleblower may assert that a claim for payment of items or services resulting from a violation of the Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil FCA. Although there are a number of statutory exemptions and regulatory safe harbors to the federal Anti-Kickback Statute protecting certain common business arrangements and activities from prosecution or regulatory sanctions, the exemptions and safe harbors are drawn narrowly. Practices that involve remuneration to those who prescribe, purchase, or recommend pharmaceutical and biological products, including certain discounts, or engaging such individuals as consultants, advisors, or speakers, may be subject to scrutiny if they do not fit squarely within an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability. Moreover, there are no safe harbors for many common practices, such as educational and research grants, charitable donations, product support and patient assistance.

The federal civil FCA prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of federal funds, knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or avoiding, decreasing, or concealing an obligation to pay money to the federal government. Actions under the FCA may be brought by the federal government or as a qui tam action by a private individual in the name of the government. Many pharmaceutical manufacturers have been investigated and have reached substantial financial settlements with the federal government under the civil FCA for a variety of alleged improper activities. The government may deem companies to have "caused" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. The civil FCA provides for treble damages and a civil penalty for each false claim, such as an invoice or pharmacy claim for reimbursement, which can aggregate into millions of dollars, and potential exclusion from federal health care programs.

The civil monetary penalties statute is another statute under which biotechnological companies may be subject to enforcement. Among other things, the civil monetary penalties statue imposes fines against any person who is determined to have knowingly presented, or caused to be presented, claims to a federal healthcare program that the person knows, or should know, is for an item or service that was not provided as claimed or is false or fraudulent.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") prohibits, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, and knowingly and willfully falsifying, concealing, or covering up a material fact or making any materially false statements or representations in connection with the delivery of, or payment for, healthcare benefits, items, or services relating to healthcare matters. The government need not establish actual knowledge of the statute, or the specific intent in order to prove a violation.

The federal Physician Payment Sunshine Act requires manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to direct or indirect payments and other transfers of value to physicians and teaching hospitals and certain other HCPs (such as physicians assistants and nurse practitioners), and ownership and investment interests held by physicians and their immediate family members, with the reported information made public on a searchable website.

Payment or reimbursement of prescription therapeutics by Medicaid or Medicare requires the product's labeler to submit certified pricing information to CMS. The Medicaid Drug Rebate statute requires labelers, as a condition of payment by Medicaid, to calculate and report price points, which are used to determine Medicaid rebate payments shared between the states and the federal government and Medicaid payment rates for certain therapeutics, to pay quarterly rebates on prescriptions paid by Medicaid, and to provide a discount based on the Medicaid rebate percentage to certain hospitals and clinics under the 340B program. For most therapeutics paid under Medicare Part B, labelers must also calculate and report their Average Sales Price, which is used to determine the Medicare Part B payment rate. In addition, therapeutics covered by Medicaid are subject to an additional inflation penalty which can substantially increase rebate payments. For products approved under a BLA (including biosimilars) or an NDA, the Veterans Health Care Act ("VHCA") requires labelers, as a condition of payment by Medicaid, to calculate and report to the Veterans Administration a different price called the Non-Federal AMP, which is used to determine the maximum price that can be charged to certain federal agencies, referred to as the Federal Ceiling Price ("FCP"). Like the

Medicaid rebate amount, the FCP includes an inflation penalty. A Department of Defense statute and regulation requires labelers to provide this discount on therapeutics dispensed by retail pharmacies when paid by the TRICARE Program, the health care program for military personnel, retirees, and related beneficiaries. All of these price reporting requirements create risk of submitting false information to the government, and potential FCA liability.

The VHCA also requires labelers of covered therapeutics participating in the Medicaid program to enter into Federal Supply Schedule contracts with the VA through which their covered therapeutics must be sold to certain federal agencies at FCP. This necessitates compliance with applicable federal procurement laws and regulations, including submission of commercial sales and pricing information, and subjects us to contractual remedies as well as administrative, civil, and criminal sanctions. In addition, the VHCA requires labelers participating in Medicaid to agree to provide different mandatory discounts to certain Public Health Service grantees and other safety net hospitals and clinics under the 340B program based on the labelers' reported Medicaid pricing information. The 340B program has its own regulatory authority to impose sanctions for non-compliance and adjudicate overcharge claims against labelers by the purchasing entities.

Further, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its respective implementing regulations imposes certain requirements on covered entities and their business associates – certain persons or entities that create, receive, maintain, or transmit protected health information in connection with providing a specified service or performing a function on behalf of a covered entity. In addition, other federal and state laws may govern the privacy and security of health and other information in certain circumstances. For example, state data breach notification laws, state health information and/or genetic privacy laws, and federal and state consumer protection and consumer privacy laws (such as Section 5 of the Federal Trade Commission Act (the "FTC Act") and the California Consumer Privacy Act, as amended by the California Privacy Rights Act (the "CCPA")) and other similar states laws differ from each other in significant ways, thus complicating compliance efforts. Any failure by us or any of our third-party service providers to follow such laws could result in significant liability or reputational harm under such state and federal privacy and other laws. The landscape of federal and state laws regulating personal data is constantly evolving, and compliance with these laws requires a flexible privacy framework and substantial resources, and compliance efforts will likely be an increasing and substantial cost in the future.

Outside the United States, our operations may implicate international data protection laws, including the EU General Data Protection Regulation 2016/679 ("EU GDPR"). The EU GDPR and the EU GDPR as incorporated into the laws of the United Kingdom ("UK GDPR," together with the EU GDPR, "GDPR") impose strict obligations and restrictions on the ability to collect, analyze, and transfer personal data, including health data and samples from clinical trials and adverse event reporting. For example, these obligations and restrictions may concern obtaining explicit consent of the individuals to whom the personal data relate, providing transparency notices to individuals, sharing personal data with third parties, transferring personal data out of the EU, reporting personal data breaches with data protection authorities and affected individuals, and ensuring the security and confidentiality of personal data. Violations of EU data protection laws may result in significant financial penalties (including possible fines of up to 4% of global annual turnover for the preceding financial year or €20 million (or €17.5 million in the UK) whichever is higher). The EU GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with data protection authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the EU GDPR. These privacy and data protection laws and regulations increase our responsibility and liability in relation to personal data that we process, and compliance has been and is expected to continue to be difficult, constantly evolving, costly and time consuming.

Moreover, as a result of the broad scale release and availability of Artificial Intelligence (AI) technologies such as generative AI, there is a global trend towards more regulation (e.g., the EU AI Act and AI laws passed in states of the United States) to ensure the ethical use, privacy, and security of AI and the data that it processes. Compliance with such laws will likely be an increasing and substantial cost in the future.

Many states have also adopted laws similar to certain of the above federal laws, which may be broader in scope and apply to items or services reimbursed by any third-party payor, including commercial insurers. Certain state laws also regulate sponsors' use of prescriber-identifiable data. Certain states also require implementation of commercial compliance programs and compliance with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; impose restrictions on marketing practices; or require drug companies to track and report information related to payments, gifts, and other items of value to physicians and other healthcare providers. Other state laws and local ordinances require identification or licensing of sales representatives.

Recently, states have enacted or are considering legislation intended to make drug prices more transparent and deter significant price increases, typically as consumer protection laws. These laws may affect our future sales, marketing, and other promotional activities by imposing administrative and compliance burdens.

If our operations are found to be in violation of any of the laws or regulations described above or any other applicable laws, we may be subject to penalties or other enforcement actions, including significant criminal, civil, and/or administrative monetary penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, corporate integrity agreements, suspension and debarment from government contracts, and refusal of orders under existing government contracts, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

Coverage and Reimbursement

The commercial success of our product candidates and our ability to commercialize any approved product candidates successfully will depend in part on the extent to which governmental payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers, and other third-party payors provide coverage for and establish adequate reimbursement levels for our product candidates. Government authorities, private health insurers, and other organizations generally decide which therapeutics they will pay for and establish reimbursement levels for healthcare. Medicare is a federally funded program managed by CMS through local fiscal intermediaries and carriers that administer coverage and reimbursement for certain healthcare items and services furnished to the elderly and disabled. Medicaid is an insurance program for certain categories of patients whose income and assets fall below state defined levels and who are otherwise uninsured that is both federally and state funded and managed by each state. The federal government sets general guidelines for Medicaid and each state creates specific regulations that govern its individual program, including supplemental rebate programs that restrict coverage to therapeutics on the state Preferred Drug List. Similarly, government laws and regulations establish the parameters for coverage of prescription therapeutics by health plans participating in state exchanges and TRICARE. Some states have also created pharmacy assistance programs for individuals who do not qualify for federal programs. In the United States, private health insurers and other third-party payors often provide reimbursement for products and services based on the level at which the government provides reimbursement through the Medicare or Medicaid programs for such products and services.

In the United States, the EU, and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which often has resulted in average selling prices lower than they would otherwise be and sometimes at or below the provider's acquisition cost. In the United States, it is also common for government and private health plans to use coverage determinations to leverage rebates from labelers in order to reduce the plans' net costs. These restrictions and limitations influence the purchase of healthcare services and products and lower the realization on labelers' sales of prescription therapeutics. Third-party payors are developing increasingly sophisticated methods of controlling healthcare costs. Third-party payors may limit coverage to specific therapeutic products on an approved list, or formulary, which might not include all of the FDA approved products for a particular indication or might impose high copayment amounts to influence patient choice. Third-party payors also control costs by requiring prior authorization or imposing other dispensing restrictions before covering certain products and by broadening therapeutic classes to increase competition. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. Absent clinical differentiators, third-party payors may treat products as therapeutically equivalent and base formulary decisions on net cost. To lower the prescription cost, labelers frequently rebate a portion of the prescription price to the third-party payors. Recently, purchasers and third-party payors have begun to focus on value of new therapeutics and sought agreements in which price is based on achievement of performance metrics.

Federal programs also impose price controls through mandatory ceiling prices on purchases by federal agencies and federally funded hospitals and clinics and mandatory rebates on retail pharmacy prescriptions paid by Medicaid and TRICARE. These restrictions and limitations influence the purchase of healthcare services and products. Legislative proposals to reform healthcare or reduce costs under government programs may result in lower reimbursement for our product candidates or exclusion of our product candidates from coverage. In addition, government programs like Medicaid include substantial penalties for increasing commercial prices over the rate of inflation which can affect realization and return on investment.

Private payors often rely on the lead of the governmental payors in rendering coverage and reimbursement determinations. Therefore, achieving favorable Medicare coverage and reimbursement is usually a significant gating issue for successful introduction of a new product. In addition, many government programs as a condition of participation mandate fixed discounts or rebates from labelers regardless of formulary position or utilization, and then rely on competition in the market to attain further price reductions, which can greatly reduce realization on the sale.

Further, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement, and utilization, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, competition within therapeutic classes, judicial decisions and governmental laws and regulations related to Medicare, Medicaid, and healthcare reform, biotechnological coverage and reimbursement policies, and pricing in general. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Sales of our product candidates will therefore depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or reimbursed by government health administration authorities, such as Medicare and Medicaid, private health insurers, and other third-party payors.

As a result of the above, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our product candidates, if approved, in addition to the costs required to obtain FDA approvals. Our product candidates may not be considered medically necessary or cost-effective, or the rebate percentages required to secure coverage may not yield an adequate margin over cost. Additionally, companies are increasingly finding it necessary to establish bridge programs to assist patients with access to new therapies during protracted initial coverage determination periods.

Moreover, a payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved or that significant price concessions will not be required to avoid restrictive conditions. High health plan copayment requirements may result in patients refusing prescriptions or seeking alternative therapies. Additionally, where a new indication has been approved for a drug or biologic previously approved under a different NDA or BLA, health plans may cover off-label use of the original drug, even if it cannot be marketed for the new indication. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in therapeutic development. Legislative action to reform healthcare or reduce costs under government insurance programs may result in lower reimbursement for our products and product candidates or exclusion of our products and product candidates from coverage. The cost containment measures that healthcare payors and providers are instituting and any healthcare reform could significantly reduce our revenues from the sale of any approved product candidates. We cannot provide any assurances that we will be able to obtain and maintain third-party coverage or adequate reimbursement for our product candidates in whole or in part.

Healthcare Reform Measures

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system. The United States government, state legislatures, and foreign governments also have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement, and requirements for substitution of generic products for branded prescription products.

In recent years, Congress has considered reductions in Medicare reimbursement levels for products administered by physicians. CMS, the agency that administers the Medicare and Medicaid programs, also has authority to revise reimbursement rates and to implement coverage restrictions for some products. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products. While Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payers.

The ACA substantially changed the way healthcare is financed by both governmental and private insurers and significantly impacts the pharmaceutical industry. The ACA is intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical manufacturers, and impose additional health policy reforms. Among other things, the ACA expanded manufacturers' rebate liability under the MDRP by increasing the minimum Medicaid rebate for both branded and generic products, expanded the 340B program, and revised the definition of AMP, which could increase the amount of Medicaid rebates manufacturers are required to pay to states. The legislation also extended Medicaid rebates, previously due only on fee-for-service Medicaid utilization, to include the utilization of Medicaid managed care organizations as well and created an alternative rebate formula for certain new formulations of certain existing products that is intended to increase the amount of rebates due on those products. CMS has issued final regulations to implement the changes to the MDRP and other changes under the ACA. Since that time, there have been significant ongoing efforts to modify or eliminate the ACA. For example, the Tax Cuts and Job Act of 2017, repealed the shared responsibility

payment for individuals who fail to maintain minimum essential coverage under section 5000A of the Internal Revenue Code of 1986, as amended, or the Code, commonly referred to as the individual mandate.

Other legislative changes have been proposed and adopted since the passage of the ACA. The Budget Control Act of 2011, among other things, resulted in aggregate reductions to Medicare payments to healthcare providers of up to 2.0% per fiscal year, which went into effect in April 2013. Subsequent legislation extended sequestration to 2031. Sequestration is currently set at 2% and will increase to 2.25% for the first half of fiscal year 2030, to 3% for the second half of fiscal year 2030, and to 4% for the remainder of the sequestration period that lasts through the first six months of fiscal year 2031. The American Taxpayer Relief Act among other things, reduced Medicare payments to several types of providers, including hospitals, imaging centers, and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. The Inflation Reduction Act of 2022 (the "IRA") contains substantial drug pricing reforms, including the establishment of a drug price negotiation program within the United States. Department of Health and Human Services that would require manufacturers to charge a negotiated "maximum fair price" for certain units of certain selected drugs or pay an excise tax for noncompliance, the establishment of rebate payment requirements on manufacturers of certain drugs payable under Medicare Parts B and D to penalize price increases that outpace inflation, and a requirement that manufacturers provide certain discounts on Part D drugs. Substantial penalties can be assessed for noncompliance with the drug pricing provisions in the IRA. The IRA could have the effect of reducing the prices we can charge and reimbursement we receive for our products, if approved, thereby reducing our profitability, and could have a material adverse effect on our financial condition, results of operations, and growth prospects. The effects of the IRA on our business and the pharmaceutical industry in general is not yet known.

In addition, legislation and regulatory actions have created certain price reporting obligations under the MDRP and 340B Program. Under the MDRP, a manufacturer is required to pay a rebate to each state Medicaid program for its covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds made available to states for its covered outpatient drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data that manufacturers report on a monthly and quarterly basis to CMS, the federal agency that administers Medicare and Medicaid. A manufacturer that becomes aware that its Medicaid reporting for a prior quarter was incorrect, or has changed as a result of recalculation of the pricing data, may be obligated to resubmit the corrected data for up to three years after those data originally were due, which revisions could affect rebate liability for prior quarters. If a manufacturer fails to pay the required rebate amount or report pricing data on a timely basis, it may be subject to civil monetary penalties and/or termination of its Medicaid Drug Rebate program agreement, in which case federal payments may not be available under Medicaid or Medicare Part B for its covered outpatient drugs. Federal law requires that any manufacturer that would like federal funds to be available to pay for its covered outpatient drugs under Medicaid or Medicare Part B to also participate in the 340B program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B program requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs. If a manufacturer is found to have knowingly and intentionally charged 340B covered entities more than the statutorily mandated ceiling price, it could be subject to significant civil monetary penalties and/or such failure also could be grounds for HRSA to terminate its agreement to participate in the 340B program, in which case its covered outpatient drugs would no longer be eligible for federal payment under the Medicaid or Medicare Part B program.

In addition to pricing regulations, reforms of regulatory approval frameworks may adversely affect our pricing strategy, if commercialized. At a federal level, President Trump reversed some of President Biden's executive orders including rescinding Executive Order 14087 entitled "Lowering Prescription Drug Costs for Americans." President Trump may issue new executive orders designed to impact drug pricing. A number of these and other proposed measures may require authorization through additional legislation to become effective. Congress and the Trump administration have indicated that they will continue to seek new legislative measures to control drug costs.

We expect that changes to the ACA, the IRA, and the Medicare and Medicaid programs, additional changes allowing the federal government to directly negotiate prices and changes stemming from other healthcare reform measures, especially with regard to healthcare access or financing or legislation in individual states, could have a material adverse effect on our business.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access, sometimes establishing Prescription Drug Affordability Boards (or similar entities) to review high-cost drugs, and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that additional federal, state, and foreign healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in limited coverage and reimbursement and reduced demand for our products, once approved, or additional pricing pressures.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act ("FCPA") prohibits any United States individual or business from paying, offering, or authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party, or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. Activities that violate the FCPA, even if they occur wholly outside the United States, can result in criminal and civil fines, imprisonment, disgorgement, oversight, and suspension and debarment from government contracts, and refusal of orders under existing government contracts. Enforcement actions may be brought by the Department of Justice or the Securities and Exchange Commission ("SEC"), and legislation has expanded the SEC's power to seek disgorgement in all FCPA cases filed in federal court and extended the statute of limitations in the SEC enforcement actions in intent-based claims such as those under FCPA from five years to ten years.

Compliance with the FCPA may require significant technical and legal expertise and capital investment, which may be costly and cannot be predicted with certainty. Criminal and/or civil violations or alleged violations of the FCPA can result in significant criminal and/or civil penalties, fines, disgorgement, exclusion from government contracts, imprisonment of current or former employees, or other sanctions. They may also result in litigation with affected parties. Any of these factors may have an adverse effect on our operations and/or reputation, which could have a material adverse effect on our business, financial condition, competitive position, and other results of operations. We are subject to other international anti-corruption laws which may entail similar risks.

Regulation Outside of the United States

In addition to regulations in the United States, we may be subject to a variety of regulations in foreign jurisdictions that govern, among other things, clinical trials and any commercial sales and distribution of our product candidates, if approved, either directly or through our distribution partners. Whether or not we obtain FDA approval for a product candidate, we must obtain the requisite approvals from regulatory authorities in foreign jurisdictions prior to the commencement of clinical trials or marketing and sale of the product in those countries. The foreign regulatory approval process includes all of the risks associated with the FDA approval process described above, and the time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Requirements governing the conduct of clinical trials, product approval, pricing, and reimbursement vary from country to country. For instance, for conducting clinical trials in the EU, Regulation (EU) No 536/2014 (*Clinical Trials Regulation*) has applied since 31 January 2022. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in other countries. Moreover, some nations may not accept clinical studies performed for the United States approval to support approval in their countries or require that additional studies be performed on natives of their countries. In addition, in certain foreign markets, the pricing of drug products is subject to government control and reimbursement may in some cases be unavailable or insufficient. Resulting prices could be insufficient to generate an acceptable return to us or any future partner of ours. If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions, and criminal prosecution.

European Union Drug Development and Approval

Clinical Trial Approval

In April 2014, the EU adopted the Clinical Trials Regulation (EU) No 536/2014, or CTR, which repealed and replaced the previous Clinical Trials Directive (2001/20/EC) on January 31, 2022. The transitory provisions of the CTR provide that all ongoing clinical trials must have transitioned to the CTR by January 31, 2025. The CTR overhauls the previous system of approvals for clinical trials in the EU. Specifically, the CTR, which is directly applicable in all EU Member States (meaning no national implementing legislation in each Member State is required), aims at simplifying and streamlining the approval of clinical trials in the EU, simplifying adverse-event reporting procedures, improving the supervision of clinical trials and increasing their transparency. The main characteristics of the CTR include: a streamlined application procedure via a single-entry point through the Clinical Trials Information System, or CTIS; a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials. The role of the relevant ethics committees in the assessment procedure continues to be governed by the national law of the applicable Member State, however overall related timelines are defined by the CTR.

Marketing Authorization

To obtain a marketing authorization for a product in the EU, an applicant must submit a MAA, either under a centralized procedure administered by the EMA or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure, or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the EU or the additional Member States of the European Economic Area (Norway, Iceland and Liechtenstein) ("EEA").

The centralized procedure provides for the grant of a single marketing authorization by the EC that is valid for all EU Member States, as well as the additional Member States of the EEA. Pursuant to Regulation (EC) No. 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (gene-therapy, somatic cell-therapy or tissue-engineered medicines) and products with a new active substance indicated for the treatment of cancer, HIV, AIDS, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions or viral diseases. The centralized procedure is optional for products containing a new active substance not yet authorized in the EU, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.

Under the centralized procedure, the Committee for Medicinal Products for Human Use ("CHMP"), established at the EMA is responsible for conducting an initial assessment of a product. The maximum timeframe for the evaluation of a MAA is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Clock stops may extend the timeframe of evaluation of an application considerably beyond 210 days. Where the CHMP gives a positive opinion, the EMA provides the opinion together with supporting documentation to the EC, who makes the final decision to grant a marketing authorization, which is issued within 67 days of receipt of the EMA's recommendation. Accelerated evaluation may be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the viewpoint of public health and, in particular, therapeutic innovation. If the CHMP accepts such a request, the time limit of 210 days will be reduced to 150 days, but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that it is no longer appropriate to conduct an accelerated assessment.

National marketing authorizations, which are issued by the competent authorities of the Member States of the EU and only cover their respective territory, are available for products not falling within the mandatory scope of the centralized procedure. Where a product has already been authorized for marketing in a Member State of the EU, this national authorization can be recognized in other Member States through the mutual recognition procedure. If the product has not received a national authorization in any Member State at the time of application, it can be approved simultaneously in various Member States through the decentralized procedure.

Data and Market Exclusivity in the European Union

In the EU, new chemical entities (including both small molecules and biological medicinal products) approved on the basis of a complete and independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. Data exclusivity, if granted, prevents generic or biosimilar applicants from referencing the innovator's pre-clinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization, for a period of eight years from the date on which the reference product was first authorized in the EU. During the additional two-year period of market exclusivity, a generic or biosimilar MAA can be submitted, and the innovator's data may be referenced, but no medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to authorization, is held to bring a significant clinical benefit in comparison with currently approved therapies. There is no guarantee that a product will be considered by the EMA to be a new chemical entity, and products may not qualify for data exclusivity. Even if a product is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company could nevertheless also market another version of the product if such company obtained marketing authorization based on a marketing authorization application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Orphan Designation and Exclusivity

Regulation (EC) No 141/2000 and Regulation (EC) No. 847/2000 provide that a product can be designated as an orphan medicinal product by the EC if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition, (2) either (i) such condition affects no more than five in ten thousand persons in the EU when the application is made, or (ii) without the benefits derived from orphan status, it is unlikely that the marketing of the product in the EU would generate sufficient return to justify the necessary investment in its development; (3) there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the product would be of significant benefit to those affected by that condition.

An orphan designation provides a number of benefits, including fee reductions, regulatory assistance and the possibility to apply for a centralized EU marketing authorization. Marketing authorization for an orphan medicinal product leads to a ten-year period of market exclusivity being granted following marketing approval of the orphan product. During this market exclusivity period, the EMA, the EC or the competent authorities of the EU Member States may only grant marketing authorization to a "similar medicinal product" for the same therapeutic indication if: (i) a second applicant can establish that its product, although similar to the authorized product, is safer, more effective or otherwise clinically superior; (ii) the marketing authorization holder for the authorized product consents to a second orphan medicinal product application; or (iii) the marketing authorization holder for the authorized product cannot supply enough orphan medicinal product. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation because, for example, the product is sufficiently profitable not to justify market exclusivity. Orphan designation must be requested before submitting an application for marketing approval. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The aforementioned EU rules are generally applicable in the EEA.

Reform of the Regulatory Framework in the European Union

The EC introduced legislative proposals in April 2023 that, if implemented, will replace the current regulatory framework in the EU for all medicines (including those for rare diseases and for children). The EC has provided the legislative proposals to the European Parliament and the European Council for their review and approval, and, in April 2024, the European Parliament proposed amendments to the legislative proposals. Once the EC's legislative proposals are approved (with or without amendment), they will be adopted into EU law.

HUMAN CAPITAL

As of February 24, 2025, we had 95 employees, of which 74 employees are located in the United States, 20 are located in India and one is located in Canada. The majority of the employees are full-time. None of our employees are represented by a labor union or covered by a collective bargaining agreement. In addition to our employees, we engage various consultants to support key areas of our business, including support of our research and development, administrative, manufacturing, and commercialization activities.

Talent Management

Our human capital is critical to the success of our mission to deliver new options for people facing serious disease and conditions. We consider the performance, skills, and intellectual capital of our employees to be an essential driver of this mission and a key to our future prospects. As such, we emphasize a number of measures and objectives in attracting, retaining, and developing our human capital, including, among others, employee safety, wellness, engagement, and compensation and pay equity. Additionally, we recognize that our employees perform best when they know how their work contributes to our overall strategy. To achieve this, we emphasize open and direct communication through the use of a variety of channels, including company-wide business updates and written communications from the leadership team.

Compensation and Benefits

Our compensation programs are designed to align our employees' interests with our achievement of our primary business goals. The salaries, bonuses, and opportunities for equity ownership provided to our employees are competitive within our industry and we engage outside compensation and benefits consulting firms to independently evaluate the effectiveness of our compensation and benefit programs and to provide benchmarking against our peers within the industry. The benefit options we provide are comprehensive and allow our employees and their families to live healthier and more secure lives. All full-time employees are eligible for medical, dental, and vision insurance, paid time off, a 401(k) plan, and group life and disability coverage.

Employee Development and Leadership

The development of our employees is critical to our success. We believe that continued learning and development is an essential part to retaining our employees and creating a culture of learning and leadership. We encourage employees to participate and to take advantage of a variety of learning and development resources that we provide, including online skills courses, professional development events, and internal and external training programs based on individual needs.

Values

We are guided by a commitment to accountability, respect, and collaboration. These principles drive the values of our employees and agents that enable us to propel the future of medical science. These values include:

- *Respect* — Acknowledging individual talents and understanding success requires everyone.
- *Integrity* — An unwavering commitment to do what is right.
- *Teamwork* — Collaboratively arriving at solutions so our people, patients, and shareholders benefit.
- *Accountability* — Taking ownership of key deliverables.

CORPORATE INFORMATION

We were originally incorporated as a Massachusetts corporation in 2000 under the name Histogenics and underwent a corporate reorganization in 2006, pursuant to which we were reincorporated as a Delaware corporation. On September 27, 2019, we completed a reverse merger (the "Merger") with Ocugen OpCo, Inc. ("OpCo") in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of April 5, 2019, by and among OpCo, Restore Merger Sub, Inc., our wholly owned subsidiary ("Merger Sub"), and us, as amended, pursuant to which Merger Sub merged with and into OpCo, with OpCo surviving as our wholly owned subsidiary. Immediately after the completion of the Merger, we changed our name to Ocugen, Inc. and the business previously conducted by OpCo became the business conducted by us. Our common stock trades on The Nasdaq Capital Market ("Nasdaq") under the symbol "OCGN."

Our principal office is located at 11 Great Valley Parkway, Malvern, Pennsylvania 19355, and our telephone number is (484) 328-4701. Our website address is www.ocugen.com. Our website and the information contained on, or that can be accessed through, our website shall not be deemed to be incorporated by reference in, and is not considered part of this Annual Report. You should not rely on any such information in making your decision whether to purchase our common stock.

AVAILABLE INFORMATION

We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Securities Exchange Act of 1934, as amended (the "Exchange Act"). The SEC maintains an internet website, www.sec.gov, that contains reports, proxy, and information statements, and other information regarding issuers, including us, that file electronically with the SEC. Copies of each of our filings with the SEC on Form 10-K, Form 10-Q, and Form 8-K and amendments to those reports, can be viewed and downloaded free of charge at our website, www.ocugen.com, as soon as reasonably practicable after the reports and amendments are electronically filed with or furnished to the SEC. Our website and the information contained on, or that can be accessed through, our website shall not be deemed to be incorporated by reference in, and is not considered part of this Annual Report.

A copy of our Corporate Governance Guidelines, Code of Business Conduct and Ethics and the charters of the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee are posted on our website, www.ocugen.com, under "Investors". Any amendments to Code of Business Conduct and Ethics are also being posted on our website, www.ocugen.com, under "Investors".

Item 1A. Risk Factors.

Careful consideration should be given to the following risk factors, together with all other information set forth in this Annual Report, including our consolidated financial statements and related notes, and "Management's Discussion and Analysis of Financial Condition and Results of Operations," and in other documents that we file with the SEC, in evaluating Ocugen, Inc. and our subsidiaries (collectively, the "Company", "we", or "our") and our business, before investing in our common stock. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The market price of our common stock could decline if one or more of these risks or uncertainties were to occur, which may cause you to lose all or part of the money you paid to buy our common stock. The risk factors described below disclose both material and other risks and are not intended to be exhaustive and are not the only risks facing us. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations. Certain statements below are forward-looking statements. See "Special Note Regarding Forward-Looking Statements" in this Annual Report.

Risk Factors Summary

Investing in our securities involves a high degree of risk. Before deciding whether to invest in our securities, you should consider carefully the risks and uncertainties described in this section of this Annual Report. These risks and uncertainties include, but are not limited to, the following:

- We have incurred significant losses and negative cash flows from operations since our inception. We may incur losses over the next several years and may never achieve or maintain profitability. These factors raise substantial doubt about our ability to continue as a going concern absent obtaining significant additional funding.
- We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce, or eliminate our product development programs or commercialization efforts.
- We will need additional capital in order to enable us to successfully develop our product candidates, and such funding may not be available on acceptable terms, or at all. Raising additional capital may cause dilution to stockholders, restrict our operations, or require us to relinquish rights to our technologies or product candidates.
- The terms of our Loan and Security Agreement with Avenue Capital Management II, L.P. and the lenders listed therein as well as our loan agreement with EB5 Life Sciences, L.P. require us to meet certain operating covenants and place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.
- We are substantially dependent on the success of our product candidates. We cannot guarantee that our product candidates will successfully complete development, receive regulatory approval, or be successfully commercialized.
- Our product candidates generated from our modifier gene therapy platform are based on a novel technology and face an uncertain regulatory environment, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, our completion of clinical trials and receipt of necessary regulatory approvals could be delayed or prevented.
- We have no prior experience in the marketing, sale, and distribution of biotechnology products and there can be no assurance that our product candidates, if approved, will be successfully commercialized.
- We face significant competition from other pharmaceutical and biotechnology companies, academic institutions, government agencies, and other research organizations. Our operating results will suffer if we fail to compete effectively.
- If third-party payors do not reimburse patients for our products candidates, if approved, or if reimbursement levels are set too low for us to sell our product candidates at a profit, our ability to successfully commercialize our product candidates, if approved, and our results of operations will be harmed.
- We rely, and expect to continue to rely, on third parties to conduct, supervise, and monitor our preclinical studies and clinical trials we may initiate, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials or failing to comply with regulatory requirements.
- If we encounter difficulties in negotiating commercial manufacturing and supply agreements with third-party manufacturers and suppliers of our product candidates or any product components, our ability to commercialize our product candidates, if approved, would be impaired.

- If the manufacturers upon whom we rely on fail to produce our product candidates or product components pursuant to the terms of contractual arrangements with us or fail to comply with stringent regulations applicable to biotechnology manufacturers, we may face delays in the development and commercialization of, or be unable to meet demand for, our product candidates, if approved, and may lose potential revenues.
- We may seek to collaborate with third parties for the development or commercialization of our product candidates. We may not be successful in establishing or maintaining collaborative relationships, any of which could adversely affect our ability to develop and commercialize our product candidates.
- We may be unable to obtain and maintain patent protection for our technology and product candidates, or the scope of the patent protection obtained may not be sufficiently broad or enforceable, such that our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and product candidates may be impaired.
- We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming, and unsuccessful.
- Certain aspects of our product candidates are protected by patents exclusively licensed from other companies or institutions. If these licensors terminate their agreements with us or fail to maintain or enforce the underlying patents or licenses thereto, or we otherwise lose our rights to these patents, our competitive position and our market share in the markets for any of our licensed and approved products will be harmed.
- Provisions in our charter documents and under Delaware law could discourage a takeover that stockholders may consider favorable and may lead to entrenchment of management.
- The trading price of the shares of our common stock could be highly volatile, and purchasers of our common stock could incur substantial losses.
- Our future success depends on our ability to retain key executives and to attract, retain, and motivate qualified personnel.
- We have restated our prior consolidated financial statements, which may lead to additional risks and uncertainties, including loss of investor confidence and negative impacts on our stock price.
- If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, investors may lose confidence in our financial reporting, and the trading price of our common stock may decline.
- The use of new and evolving technologies, such as artificial intelligence, in our business may result in spending material resources and presents risks and challenges that can impact our business including by posing security and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability.

Risks Related to Our Financial Position and Capital Requirements

We have incurred significant losses and negative cash flows from operations since our inception. We may incur losses over the next several years and may never achieve or maintain profitability. These factors raise substantial doubt about our ability to continue as a going concern absent obtaining significant additional funding.

Since inception, we have incurred significant net losses and may continue to incur net losses in the future. Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern for the next 12 months from the date of the consolidated financial statements included in this Annual Report are issued. As a result, our independent public accounting firm included an explanatory paragraph regarding the same in its report on this Annual Report. Substantial doubt about our ability to continue as a going concern may create negative reactions to the price of our common stock and we may have a more difficult time obtaining financing in the future as a result.

We have not generated significant revenue to date and have funded our operations to date through the sale of common stock, warrants to purchase common stock, the issuance of convertible notes and debt, and grant proceeds. We incurred net losses of approximately $54.1 million and $63.1 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $340.2 million and a cash balance of $58.5 million. This amount will not meet our capital requirements over the next 12 months. We estimate that our cash and cash equivalents will enable us to fund our operations into the first quarter of 2026. Based on this estimate, we will need to raise significant additional capital in order to fund our future operations. We have based this estimate on assumptions that may prove to be wrong, and our operating and capital requirements may change as a result of many factors currently unknown to us.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, is not available in sufficient amounts, or we do not have sufficient authorized shares, we may be required to delay, limit, or eliminate the development of business opportunities and our ability to achieve our business objectives, our competitiveness, and our business, financial condition, and results of operations will be materially adversely affected. In addition, economic circumstances outside of our control such as a recession or depression and inflation may reduce our ability to access capital, which could negatively affect our liquidity and ability to continue as a going concern. Further, the perception that we may not be able to continue as a going concern may cause others to choose not to do business with us due to concerns about our ability to meet our contractual obligations.

To date, we have not generated any revenues from the sale of products, and absent the realization of sufficient revenues from product sales, if any, of our current or future product candidates, we may never attain profitability in the future. To date, we have devoted substantially all of our financial resources and efforts to research and development, including preclinical and clinical studies. We may continue to incur losses from operations in the next several years as we increase our expenditures in research and development in connection with our ongoing and planned clinical trials and other development and pre-commercialization activities. Even if we obtain a regulatory approval to market a product candidate, our future revenues will depend upon the size of any markets in which our product candidates have received such approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payors, and adequate market share for our products in those markets.

We anticipate that our expenses will increase in fiscal year 2025 compared to fiscal year 2024 as we continue to conduct clinical activities with respect to our product candidates, including the continuation of several clinical trials for our product candidates, as well as increased headcount, including management personnel to support our research and development, clinical, and business activities, and expanded infrastructure, among other factors.

Due to the inherently unpredictable nature of preclinical and clinical development and the numerous risks and uncertainties associated with such activities, we are unable to predict with any certainty the nature or amounts of the costs we will incur, the timelines we will require in our continued development efforts or the timing, or if we will be able to achieve profitability.

Additionally, our expenses will also increase if, and, as we:

- initiate preclinical studies and clinical trials for any additional product candidates that we may pursue in the future, particularly if there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates;
- seek marketing approvals for product candidates that successfully complete clinical development;
- establish sales, marketing, and distribution capabilities for our product candidates for which we obtain a regulatory approval;

- scale up our manufacturing processes and capabilities to support our clinical trials of our product candidates and commercialization of any of our product candidates for which we obtain a regulatory approval;
- expand our operational, financial, and management systems and increase personnel, including personnel to support our clinical development, manufacturing, and commercialization efforts, and our operations as a public company;
- acquire other companies, products, product candidates, or technologies, or in-license the rights to other products, product candidates, or technologies; and
- develop, maintain, expand, and protect our intellectual property portfolio.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate revenue that is sufficient to achieve profitability unless and until we obtain marketing approval for and commercialize one of our product candidates and even if we obtain marketing approval for and commercialize one of our product candidates, we may never become profitable. Our product candidates are in various stages of preclinical and clinical development or pre-commercialization, and it is unknown whether our near-term efforts to obtain regulatory approval or commercial sales may be successful or whether additional preclinical, clinical, or manufacturing data may be needed before we obtain regulatory approval for any candidate. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become profitable or inability to remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, continue or undertake commercialization efforts, diversify our product offerings, or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We have no history of commercializing pharmaceutical products, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a biotechnology company and investment in biotechnological product development is a highly speculative endeavor. Biotechnology product development entails substantial upfront capital expenditures and there is significant risk that any potential product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, to gain any required regulatory approvals or to become commercially viable. To date, our operations have been limited to organizing and staffing our company, acquiring rights to intellectual property, business planning, raising capital, and developing our product candidates. We have not yet demonstrated an ability to obtain marketing approvals, manufacture a commercial-scale product, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

We have encountered, and will continue to encounter, risks and difficulties frequently experienced by growing companies in a rapidly developing and changing industry, such as the biotechnological industry, including challenges in forecasting accuracy, determining appropriate investments of our limited resources, gaining market acceptance of our products, if approved, managing a complex regulatory landscape, and developing new product candidates. Our current operating model may require changes in order for us to scale our operations efficiently. We will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. You should consider our business and prospects in light of the risks and difficulties we face as a company focused on developing products in the fields of biopharmaceuticals and biotechnology.

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

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce, or eliminate our product development programs or commercialization efforts.

We expect to devote substantial financial resources to our ongoing and planned product development activities, particularly as we continue the development of and seek marketing approval for our product candidates and any potential future product candidates, as applicable. As of December 31, 2024, we had cash of approximately $58.5 million. This amount will not meet our capital requirements over the next 12 months. We estimate that our cash and cash equivalents will enable us to fund our operations into the first quarter of 2026. Based on this estimate, we will need to raise significant additional capital in order to fund our future operations. We have based this estimate on assumptions that may prove to be wrong, and our operating and capital requirements may change as a result of many factors currently unknown to us.

Conducting preclinical testing and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete. We cannot predict when we will be able to generate the necessary data or results required to obtain regulatory approval of products with the market potential sufficient to enable us to achieve profitability, if ever. Accordingly, we may need to obtain substantial additional funding in connection with our continuing operations. Our future capital requirements will depend on many factors, including:

- the initiation, progress, timing, costs, and results of clinical trials for our product candidates;
- the outcome, timing, and cost of the regulatory approval process for our product candidates;
- the costs of manufacturing and commercialization;
- the costs related to doing business internationally with respect to the development and commercialization of our product candidates;
- the cost of filing, prosecuting, defending, and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us;
- the costs of expanding infrastructure to support our development, commercialization, and business efforts, including the costs related to the development of a laboratory and manufacturing facility;
- the costs involved in recruiting and retaining skilled personnel;
- the extent to which we in-license or acquire other products, product candidates, or technologies;
- the extent to which we out-license our product candidates; and
- the impact of geopolitical turmoil, macroeconomic conditions, social unrest, political instability, terrorism, or other acts of war.

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. Moreover, adequate additional financing may not be available to us on acceptable terms, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce, or terminate preclinical studies, clinical trials, or other development activities for one or more of our product candidates or delay, limit, reduce, or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates.

Due to our limited resources and access to capital, we must, and have in the past decided to, prioritize development of certain product candidates over other potential product candidates. These decisions may prove to have been wrong and may adversely affect our ability to develop our own programs, our attractiveness as a commercial partner and may ultimately have an impact on our commercial success.

Because we have limited resources and access to capital to fund our operations, we must decide which product candidates to pursue and the amount of resources to allocate to each. Our decisions concerning the allocation of research, collaboration, management, and financial resources toward particular proprietary molecules in our library, product candidates or therapeutic areas may not lead to the development of viable commercial products and may divert resources away from better opportunities. Similarly, our decisions to delay, terminate or collaborate with third parties in respect of certain product development programs may also prove not to be optimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the market potential of our product candidates or misread trends in the biopharmaceutical industry, in particular for our lead product candidates, our business, financial condition and results of operations could be materially adversely affected.

We will need additional capital in order to enable us to successfully develop our product candidates, and such funding may not be available on acceptable terms, or at all. Raising additional capital may cause dilution to stockholders, restrict our operations, or require us to relinquish rights to our technologies or product candidates.

We will need additional capital in order to enable us to successfully develop and obtain authorization or approval for our product candidates. Such funding may not be available on acceptable terms, or at all. We expect to raise additional capital through public and private placements of equity and/or debt, payments from potential strategic research and development arrangements, sales of assets, government grants, licensing and/or collaboration arrangements with pharmaceutical companies or other institutions, funding from the government, or funding from other third parties. We also intend to work closely with government agencies to obtain funding for the development of our novel inhaled mucosal vaccine platform.

We are opportunistically pursuing vaccines development. In October 2023, the NIAID selected OCU500, a mucosal vaccine candidate specifically targeted to fight COVID-19, for inclusion in a Phase 1 trial comparing the administration of OCU500 via different mucosal routes—inhalation into the lungs or up the nose via a nasal spray. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

If we raise additional funds through collaborations, strategic alliances, licensing arrangements, or marketing and distribution arrangements, 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. Such arrangements may require us to grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market on our own.

The terms of our Loan and Security Agreement with Avenue Capital Management II, L.P. and the lenders listed therein as well as our loan agreement (the "EB-5 Loan Agreement") with EB5 Life Sciences, L.P. ("EB-5 Life Sciences") require us to meet certain operating covenants and place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.

On November 6, 2024, we entered into a Loan and Security Agreement with Avenue Capital Management II, L.P., as administrative agent and collateral agent (the "Agent," together with Avenue I and Avenue II, "Avenue"), Avenue Venture Opportunities Fund II, L.P., as a lender ("Avenue 2"), and Avenue Venture Opportunities Fund, L.P., as a lender ("Avenue 1", and together with Avenue 2, the "Lenders") (the “Loan and Security Agreement”) that is secured by a lien on all of our assets. As of December 31, 2024, we had $30.0 million of outstanding principal borrowings under the Loan and Security Agreement. Additionally, as of December 31, 2024, we had $2.5 million of outstanding principal borrowings under the EB-5 Loan Agreement, which we are required to repay on the seventh anniversary of the date of the last disbursement under the EB-5 Loan Agreement (unless terminated earlier pursuant to the terms of the EB-5 Loan Agreement).

The Loan and Security Agreement contains customary affirmative and negative covenants and events of default. Affirmative covenants include, among others, covenants requiring us to protect and maintain our intellectual property and comply with all applicable laws, deliver certain financial reports and maintain insurance coverage. Negative covenants include, among others, covenants restricting us from transferring any part of our business or intellectual property, incurring additional indebtedness, engaging in mergers or acquisitions, repurchasing shares, paying dividends or making other distributions, making investments, and creating other liens on our assets, including our intellectual property, in each case subject to customary exceptions. Our obligations under the EB-5 Loan Agreement are secured by substantially all of our assets other than our intellectual property. We could in the future incur additional indebtedness beyond our borrowings under the EB-5 Loan Agreement. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility. These restrictions may include, among other things, limitations on the incurrence of additional debt and specific restrictions on the use of our assets, as well as prohibitions on our ability to create liens, pay dividends, redeem capital stock or make investments. If we default under the terms of the Loan and Security Agreement, the EB-5 Loan Agreement or any future debt facility, Avenue or EB-5 Life Sciences may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Further, if we were to be liquidated, Avenue’s and EB-5 Life Sciences' rights to repayment would be senior to the rights of the holders of our common stock. Avenue could declare an event of default upon the occurrence of any event that could reasonably be expected to result in what they interpret as a material adverse effect as defined under the Loan and Security Agreement or the EB-5 Loan Agreement. Any declaration by Avenue of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline.

Our existing or future debt could have significant adverse consequences, including:

- requiring us to dedicate a substantial portion of cash flow from operations or cash on hand to the payment of interest on, and principal of, our debt, which will reduce the amounts available to fund working capital, capital expenditures, product development efforts, and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry, and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing (for instance, the EB-5 Loan Agreement includes restrictive covenants related to, among other things, the disposition of our property, the incurrence by us of any additional indebtedness, and the creation by us of any liens or other encumbrances); and
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry; and placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

In order to satisfy our current and future debt service obligations, we will be required to raise funds from external sources. We may be unable to arrange for additional financing to pay the amounts due under our existing debt. Funds from external sources

may not be available on acceptable terms, if at all. Our failure to satisfy our current and future debt obligations could adversely affect our financial condition and results of operations.

Our management has broad discretion in the use of the net proceeds from our capital raises, including our November 2024 debt financing transaction, and may not use these proceeds effectively.

Our management has broad discretion in the application of the net proceeds from our capital raises, including our July 2024 Public Offering (as defined below), November 2024 debt financing, and our stockholders will not have the opportunity as part of their investment decision to assess whether the net proceeds from our capital raises are being used appropriately. Our stockholders may not agree with our decisions, and our use of the proceeds may not yield any return on investment for our stockholders. Because of the number and variability of factors that will determine our use of the net proceeds from our capital raises, their ultimate use may vary substantially from their currently intended use. Our failure to apply the net proceeds of our capital raises effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of those net proceeds. Our stockholders will not have the opportunity to influence our decisions on how to use our net proceeds from our capital raises. We have and may continue to invest the net proceeds from our capital raises in investment-grade, interest-bearing instruments and the United States government agency securities and treasuries. These investments are not likely to yield a significant return. Our management might not apply our existing cash and cash equivalents in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business.

We have restated in the past our prior consolidated financial statements, which may lead to additional risks and uncertainties, including loss of investor confidence and negative impacts on our stock price.

In the 2023 Annual Report, we restated our consolidated financial statements as of and for the fiscal year ended December 31, 2022, and the quarters ended September 30, 2022, June 30, 2022, March 31, 2022, September 30, 2023, June 30, 2023, and March 31, 2023 (the "Restated Periods"). The determination to restate the financial statements for the Restated Periods was made by our Audit Committee of the Board upon management's recommendation following the identification of errors related us not appropriately accounting for the estimated non-cash consideration and expense in one of our collaboration arrangements. These identified errors resulted in a restatement of the following financial statements line items captions: Collaborative arrangement revenue, Research and development expenses, Other income (expense), net and Accrued expense and other current liabilities. Our management, after consultation with our prior independent registered accountants, concluded that our Previously Issued Financial Statements for the Restated Periods should no longer be relied upon.

The restatement of our Previously Issued Financial Statements has been time-consuming and expensive and could expose us to additional risks that could materially adversely affect our financial position, results of operations and cash flows, including unanticipated costs for accounting and legal fees in connection with or related to the restatement and the risk of potential stockholder litigation. If lawsuits are filed, we may incur additional substantial defense costs regardless of the outcome of such litigation. Likewise, such events might cause a diversion of our management's time and attention. If we do not prevail in any such litigation, we could be required to pay substantial damages or settlement costs. In addition, the restatement may lead to a loss of investor confidence and have negative impacts on the trading price of our common stock.

Our ability to utilize our tax net operating losses is uncertain.

We have incurred significant net operating losses since our inception. As of December 31, 2024, we had United States federal net operating loss carryforwards of approximately $263.9 million. Our ability to utilize these net operating losses to offset future tax liabilities depends on the successful development of our product candidates and future financial performance.

Additionally, our net operating losses may be subject to Section 382 of the Internal Revenue Code of 1986, as amended ("Section 382"). Generally, if an ownership change occurs within three years of the closing date of an entity's most recent change in control transaction, any existing net operating losses and certain built-in losses would be subject to an additional limitation, pursuant to Section 382. Change in control as defined by Section 382 occurs when there is an ownership change among stockholders owning directly or indirectly 5% or more of our common stock, as well as an aggregate ownership change with respect to such stockholders of more than 50% of our common stock. We have not yet conducted a comprehensive study to assess whether a change of ownership as defined by Section 382 has occurred since our inception. If it is determined that we are unable to use our net operating losses to reduce future tax liabilities, our financial condition, results of operations, and cash flows may be adversely affected.

We may be subject to future changes in tax legislation or exposure to additional tax liabilities that may adversely affect our financial condition, results of operations, and cash flows.

We are subject to taxes in the United States as well as the foreign jurisdictions where our subsidiaries are organized. Due to economic and political conditions, tax rates, tax laws, and other non-tax legislation, we may experience significant impacts as a result of prospective changes. Our future effective tax rates may be affected by changes in the valuation of deferred tax assets and liabilities, changes in available tax credits or tax deductions, as well as changes in tax law and other non-tax laws, or their interpretation.

Our tax returns and other tax matters are subject to examination by applicable tax authorities and governmental bodies. We regularly assess the likelihood of an adverse outcome resulting from examination, in order to determine any resulting impact to our provision for income taxes or deferred tax balances. There can be no assurance as to the outcome of these examinations. As such, if we were to sustain an adjustment as a result of a tax examination in excess of amounts previously accrued, our financial condition could be adversely affected.

Risks Related to Our Business and the Development of Our Product Candidates

We are substantially dependent on the success of our product candidates. We cannot guarantee that our product candidates will successfully complete development, receive regulatory approval, or be successfully commercialized.

We have invested a significant portion of our efforts and financial resources in the development of our product candidates. We currently have no products authorized or approved for which we have successfully commercially distributed, and we have not generated revenues from sales of any products. Our business and our ability to generate revenues in the near term depends entirely on the successful development, approval, and commercialization of our product candidates, which may never occur. If the results or timing of regulatory filings, the regulatory process, regulatory developments, clinical trials or preclinical studies, or other activities, actions, or decisions related to our product candidates do not meet our or others' expectations, the market price of our common stock could decline significantly.

Our product candidates are susceptible to the risks of failure inherent at any stage of product development, including the appearance of unexpected or unacceptable adverse events or failure to demonstrate efficacy in clinical trials. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials, and our product candidates may not be successfully commercialized even if they receive regulatory approval.

Our product candidates are in various stages of development ranging from pre-clinical development to pre-commercialization.

The success of our product candidates and our ability to generate revenues from our product candidates, if approved, will depend on many factors including our ability to:

- complete and obtain favorable results from our clinical trials and preclinical studies with respect to our product candidates;
- apply for and receive marketing approval from the applicable regulatory authorities;
- receive regulatory approval for claims that are necessary or desirable for successful marketing;
- receive approval for our manufacturing processes and facilities from the applicable regulatory authorities;
- recruit and enroll qualified patients for clinical trials with respect to our product candidates in a timely manner;
- expand and maintain a workforce of experienced scientists and others with experience in relevant technologies to continue to develop our product candidates;
- hire, train, and deploy marketing and sales representatives or contract with a third-party for marketing and sales representatives to commercialize product candidates in the United States and key foreign markets;
- launch and create market demand for our product candidates, if approved, through marketing and sales activities, and any other arrangements to promote these product candidates that we may otherwise establish;
- achieve market acceptance of our product candidates by patients, the medical community, and third-party payors;
- effectively compete with other therapies and establish a market share;
- maintain a continued acceptable safety and efficacy profile of our product candidates, if approved, following commercial launch;

- achieve appropriate reimbursement, pricing, and payment coverage for our product candidates, if approved;
- manufacture product candidates in sufficient quantities and at acceptable quality and manufacturing cost to meet commercial demand at launch and thereafter;
- establish and maintain agreements with wholesalers, distributors, and group purchasing organizations on commercially reasonable terms;
- pursue partnerships with, or offer licenses to, qualified third parties to promote and sell product candidates, if approved, in domestic and key foreign markets where we receive marketing approval;
- develop our product candidates for additional indications or for use in broader patient populations;
- maintain patent and trade secret protection and regulatory exclusivity for our product candidates; and
- qualify for, identify, register, maintain, enforce, and defend intellectual property rights and claims covering our products and intellectual property portfolio; and not infringe on others' intellectual property rights.

To the extent we are not able to do any of the foregoing, our business may be materially harmed. If we do not receive FDA or other applicable foreign regulatory approval for, and successfully commercialize our product candidates, we will not be able to generate revenue from these product candidates in the United States or other key foreign markets for the foreseeable future or at all.

Our product candidates generated from our modifier gene therapy platform are based on a novel technology and face an uncertain regulatory environment, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.

A substantial portion of our product research and development efforts is centered around our modifier gene therapy platform. The regulatory approval and successful commercialization of OCU400, OCU410, and OCU410ST depend on the successful development of this platform. There can be no assurance that any development problems we experience in the future related to our modifier gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. The clinical trial requirements of the FDA, the EMA, and other regulatory agencies, and the criteria used by these regulators to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as these can be more expensive and take longer than for other, better known, or extensively studied pharmaceuticals or other product types.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. For example, the FDA established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research ("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. Gene therapy clinical trials conducted at institutions that receive funding for recombinant deoxyribonucleic acid ("DNA") research from the NIH are also subject to review by the NIH Novel and Exceptional Technology and Research Advisory Committee ("NExTRAC"), formerly the Recombinant DNA Advisory Committee, which now focuses on emerging areas of research including, but not restricted to, technologies surrounding advances in recombinant or synthetic nucleic acid research. Although the FDA decides whether individual gene therapy protocols may proceed, it is possible the NExTRAC review process, if undertaken for any of our candidates, could delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Before a clinical trial can begin at a study site, the institution's IRB and its IBC have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates, or lead to significant post-approval limitations or restrictions. As we advance our gene therapy product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our gene therapy product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected for orphan ophthalmology 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.

Existing data on the safety and efficacy of gene therapy is very limited and sometimes include historically poor clinical efficacy of previous non-replicating gene therapy products. In addition, there have been publicized safety issues associated with previous gene therapy products in third-party clinical trials, including patient deaths. The results of preclinical and clinical trials performed for our product candidates will not definitively predict safety or efficacy in humans. OCU400, OCU410, and OCU410ST use an AAV vector. Possible serious side effects of other viral vector-based gene therapies in general include uncontrolled viral infections and the development of cancer, particularly lymphoma or leukemia. The risk of insertional mutagenesis or oncogenesis remains a significant concern for gene therapy, and we cannot provide any assurance that it will not occur in any of our ongoing or planned clinical trials with respect to our product candidates based on our modifier gene therapy platform. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. Potential procedure-related adverse reactions, including inflammation, can also occur. If any such adverse events occur during clinical trials, further advancement of such clinical trials could be halted or delayed, which would have a material adverse effect on our business and operations.

Finally, the public's attitude may be influenced by claims that gene therapy technology is unsafe, unethical, or immoral. If we are unable to convincingly demonstrate the safety and efficacy of our product candidates arising from our gene modifier platform, our product candidates, even if approved by the FDA or foreign regulatory authorities, may not gain the acceptance of the public or the medical community. For example, in November 2023, the FDA announced it was investigating the risk of T cell malignancies associated with currently approved BCMA- and CD19-directed autologous CAR T cell immunotherapies. The FDA subsequently announced class safety labeling changes to highlight these risks and recommend life-long monitoring of patients and clinical trial participants receiving treatment with these products. It is unclear these safety events and resulting FDA action may adversely impact the FDA's approach to regulating any product candidates we may develop based on the use of our modifier gene therapy platform, or may negatively impact patients', providers', or public perception of such product candidates.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining required regulatory approvals, we will not be able to commercialize our product candidates as expected, and our ability to generate revenue will be materially impaired.

The research, testing, manufacturing, labeling, approval, selling, marketing, and distribution of pharmaceutical products are subject to extensive regulations by the FDA and other regulatory authorities, which regulations differ from country to country. The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials. The outcome of the approval process is inherently uncertain and depends upon numerous factors, including the substantial discretion of the regulatory authorities. This is especially true for rare and/or complicated diseases. Failure can occur at any time during the clinical trial process. We cannot predict if or when we might receive regulatory approvals for any of our product candidates currently under development. Any delay in our obtaining or our failure to obtain required approvals could materially adversely affect our ability to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy for that indication. We may be unable to design and execute a clinical trial to support marketing approval. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of, manufacturing facilities and clinical trial sites by the regulatory authorities. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical, or other studies. The number and types of preclinical studies and clinical trials that will be required for regulatory approval also varies depending on the product candidate, the disease or condition that the product candidate is designed to address, and the regulations applicable to any particular product candidate. The FDA or other similar regulatory authorities may determine that our product candidates are not effective or only moderately effective (e.g., studies may not produce the necessary result on all study endpoints), that our studies failed to reach the necessary level of statistical significance, or that our product candidates have undesirable or unintended side effects, toxicities, or other characteristics that preclude us from obtaining marketing approval or prevent or limit commercial use.

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

- regulators, including the FDA and the NIH, or IRBs or IBCs 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, IRBs, or IBCs 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 CDMOs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials, or be lost to follow-up at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or the clinical trial protocol, or 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;
- us, the regulators, IRBs, or IBCs 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 policies or regulations, or changes in or the enactment of additional statutes or regulations, during the development period rendering our data insufficient to obtain marketing approval and requiring us to conduct additional studies;
- the cost of clinical trials of our product candidates may be greater than we anticipate or we may have insufficient funds for a clinical trial or to pay the substantial user fees required by the FDA upon the filing of a marketing application;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- we may have delays in adding new investigators or clinical trial sites, or we may experience a withdrawal of clinical trial sites;
- patients that enroll in our studies may misrepresent their eligibility or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the study, increase the needed enrollment size for the study, or extend the study's duration;
- 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 foreign 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 submissions in foreign jurisdictions, or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign 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.

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, if approved, 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 approved, and may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays in clinical trials may ultimately lead to the denial of marketing approval of any of our product candidates. If any of this occurs, our business, financial condition, results of operations, and prospects will be materially harmed.

The failure to comply with FDA and comparable foreign regulatory requirements may, either before or after product approval, if any, subject us to administrative or judicially imposed sanctions, including:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on our products, manufacturers, or manufacturing process;
- warning letters, Form 483s, or untitled letters alleging violations;
- civil and criminal penalties;
- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions, or import bans;
- voluntary or mandatory product recalls and publicity requirements;
- total or partial suspension of production;
- imposition of restrictions on operations, including costly new manufacturing requirements; and
- refusal to approve pending marketing applications or supplements to approved marketing applications.

Even if we were to obtain regulatory approval of a product candidate, the FDA or comparable foreign regulatory authorities may grant approval for fewer or more limited indications, populations, or uses than we request, may require significant safety warnings, including black box warnings, contraindications, and precautions, may grant approval contingent on the performance of costly post-marketing clinical trials, surveillance, restrictions on use or other requirements, including a REMS to monitor the safety or efficacy of the product, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of these scenarios could compromise the commercial prospects for our product candidates.

The development and manufacture of biologics is a complex process and entails particular risks.

OCU200 is our novel biologic designed to treat retinal diseases. The process of developing and manufacturing biologics is complex, highly regulated, and subject to multiple risks, and we have no experience in successfully developing, manufacturing, or commercializing a biologics product. The manufacturing of biologics is highly susceptible to product loss due to contamination, equipment failure, improper installation or operation of equipment, vendor or operator error, inconsistency in yields, variability in product characteristics, and difficulties in scaling the production process. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions, and higher costs.

The raw materials required in our third-party vendors' manufacturing processes are derived from biological sources. We cannot assure you that our third-party vendors have, or will, be able to obtain on commercially reasonable terms, or at all, sufficient rights to these materials derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall. If microbial, viral, or other contaminations are discovered at the facilities of our manufacturer, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials, result in higher costs of drug product, and adversely harm our business. A material shortage, recall, or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the clinical and commercial manufacturing of our product candidates, which could materially and adversely affect our operating results and development timelines. In addition, the United States government has imposed a 10% additional tariff on imports from China and may impose more restrictions on goods, including biologically derived substances, manufactured in or imported from China or impose other restrictions on companies' ability to work with Chinese biotech companies. This could have a material adverse effect on our business and operations. In addition, regional or single-source dependencies may in some cases accentuate these risks. For example, the pharmaceutical industry generally, and in some instances us or our collaborators or other third parties on which we rely, depend on China-based suppliers or service providers for certain raw materials, goods, including biologically derived substances, products and services, or other activities. Our ability or the ability of our collaborators or such other third parties to continue to engage these China-based suppliers or service provides and Chinese biotech companies for certain preclinical research programs and clinical development programs could be restricted due to geopolitical developments between the United States and China, including as a result of the escalation of tariffs or other trade restrictions or if the previously proposed federal legislation known as the BIOSECURE Act or a similar law were to be enacted.

In addition, our biologic product candidates may expose us to additional potential product liability claims. The development of biologic products entails a risk of additional product liability claims because of the risk of transmitting disease to human recipients, and substantial product liability claims may be asserted against us as a result.

OCU400 and OCU410ST have received ODDs from the FDA, OCU400 has received OMPD from the EC and OCU410ST has OMPD from the EMA. However, there is no guarantee that we will be able to maintain these designations, receive these designations for any of our other product candidates, or receive or maintain any corresponding benefits, including periods of exclusivity.

We have obtained from the FDA Office of Orphan Products, ODDs for OCU400 for RP and LCA and had previously received ODDs for the treatment of the following disease genotypes: NR2E3, *RHO*, *CEP290*, and *PDE6β* mutation-associated inherited retinal degenerations. OCU400 has additionally received OMPD from the EC, based on the recommendation of the EMA, for RP and LCA. We have also received ODD and OMPD for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt, RP19 and CORD3 diseases. We may also seek ODD or OMPD for our other product candidates, as appropriate. While these ODDs and OMPDs provide us with certain advantages, they neither shorten the development time or regulatory review time of a product candidate nor give the product candidate any advantage in the regulatory review or approval process.

Generally, if a product candidate with ODD or OMPD is the first to receive marketing approval for the orphan indication, the product is entitled to a period of marketing exclusivity, which precludes the FDA or EC from approving another MAA for the same (or a similar in the European Union) drug or biologic for the same indication for a specified time period. The applicable period is seven years in the United States and 10 years in the European Union. The European Union exclusivity period can be reduced to six years if, at the end of the fifth year, it is established that a product no longer meets the criteria for OMPD or if the product is sufficiently profitable so that market exclusivity is no longer justified. Legislation has been proposed by the EC that, if implemented, has the potential in some cases to shorten the ten-year period of orphan drug exclusivity.

We may not be able to obtain any future ODDs or OMPDs that we apply for. Receiving ODDs or OMPDs 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 ODDs or OMPDs that we receive. For instance, ODDs may be revoked if the FDA finds that the request for designation contained an untrue statement of a 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, even if we are able to receive and maintain ODDs or OMPDs, 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 or EC regulatory approval is different than the ODD or OMPD. Orphan exclusivity may also be lost for the same reasons that ODD or OMPD may be lost.

Even if we obtain orphan exclusivity for any of our current or future product candidates, that exclusivity may not effectively protect the product candidate, if approved, 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 EC can also subsequently approve a product containing the same principal molecular features for the same condition if: (i) the regulatory authority concludes that the latter product is clinically superior by means of greater effectiveness, greater safety, or providing a major contribution to patient care, (ii) if we are unable to assure a sufficient quantity of the product to meet the needs of patients with the rare disease or condition, or (iii) we consent to the authorization of the latter product.

If another sponsor receives approval for such product before we do, we would be prevented from launching our product for the orphan indication during the period of marketing exclusivity unless we can demonstrate clinical superiority to the authorized orphan product (or in other very limited circumstances).

We have pursued, and may in the future pursue, fast track, breakthrough therapy, or RMAT designations from the FDA for one or more of our product candidates. Even though some of our product candidates received RMAT Designation and these or other product candidates may in the future receive fast track, breakthrough therapy or RMAT designations, we may be unable to obtain and maintain the benefits associated with such designations. These designations may not lead to a faster development or regulatory review or approval process and will not increase the likelihood that such product candidates will receive marketing approval.

In May 2022, the FDA granted RMAT designation to NeoCart for the repair of full-thickness lesions of the knee cartilage in adults. In December 2023, the FDA granted RMAT designation to OCU400 for the treatment of RP associated with NR2E3 and *RHO* mutations. In the future, we may seek additional product designations, such as breakthrough therapy and additional

RMAT and fast track designations, which are intended to facilitate the development or regulatory review or approval process for product candidates. Receipt of such a designation is within the discretion of the FDA. Accordingly, 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.

The FDA may determine that our product candidates have undesirable side effects that could delay or prevent their regulatory approval or commercialization. If such side effects are identified during the development of our product candidates, we may need to abandon our development of such product candidates.

Undesirable side effects caused by our product candidates could cause us, IRBs, and other reviewing entities or regulatory authorities to interrupt, delay, or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. For example, if concerns are raised regarding the safety of one of our product candidates as a result of undesirable side effects identified during preclinical or clinical testing, the FDA may order us to cease further development or issue a letter requesting additional data or information prior to making a final decision regarding whether or not to approve the product candidate. FDA requests for additional data or information can result in substantial delays in the approval of a new product candidate.

Undesirable side effects caused by any unexpected characteristics (alone or in combination with other products) for any of our product candidates could also result in denial of regulatory approval by the FDA or other comparable foreign 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, 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 prevent us from commercializing and generating revenues from the sale of our product candidates.

Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound. In addition, adverse events which had initially been considered unrelated to the study treatment may later be found to be caused by the study treatment. Moreover, incorrect or improper use of our product candidates (including use more frequently than is prescribed), if approved, by patients could cause unexpected side effects or adverse events. There can be no assurance that our product candidates, if approved, will be used correctly, and if used incorrectly, such misuse could prevent our receipt or maintenance of marketing approval, resulting in label changes or regulatory authority safety communications or warnings, or hamper commercial adoption of our product candidate, if approved, at the rate we currently expect. Additionally, we currently provide expanded access to OCU400 in the United States to certain eligible adults. There may be adverse events arising during the course of this or any future EAP we might implement which could adversely impact the further development or approvability of our product candidates.

If any of our product candidates are associated with SAEs, undesirable side effects, or have properties that are unexpected, we may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe, or more acceptable from a risk-benefit perspective. The therapeutic-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. We may also be held liable for harm caused to patients and our reputation may suffer. Any of these occurrences may significantly harm our business, financial condition, results of operations, and prospects.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our completion of clinical trials and receipt of necessary regulatory approvals could be delayed or prevented.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates. Our ongoing clinical trials could be discontinued early if they experience slow enrollment, and we may also experience similar difficulties in future clinical trials. If patients are unwilling to participate in our clinical trials because of negative publicity from adverse events related to vaccines, gene therapy, or in the industry more broadly, in the clinical trials for related third party product candidates, or for other reasons, including competitive clinical trials 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 product candidates, or termination of the clinical trials altogether.

We or our clinical trial sites may not be able to identify, recruit, and enroll a sufficient number of patients, or those with the required or desired characteristics in a clinical trial, to complete our clinical trials in a timely manner. Patient enrollment is affected by other factors including:

- the size and nature of the patient population (for instance, we are pursuing clinical trials for certain orphan indications, for which the size of the patient population is limited);
- the severity of the disease under investigation;
- the existence of current treatments for the indications for which we are conducting clinical trials;
- the eligibility criteria for and design of the clinical trial in question, including factors such as frequency of required assessments, length of the study, and ongoing monitoring requirements;
- the perceived risks and benefits of the product candidate, including the potential advantages or disadvantages of the product candidate being studied in relation to other available therapies;
- competition in recruiting and enrolling patients in clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- effectiveness of publicity created by clinical trial sites regarding the trial;
- patients' ability to comply with the specific instructions related to the trial protocol, proper documentation, and use of the product candidate;
- an inability to obtain or maintain patients' informed consents;
- the risk that enrolled patients will drop out before completion or not return for post-treatment follow-up;
- the ability to monitor patients adequately during and after treatment;
- the ability to compensate patients for their time and effort; and
- the proximity and availability of clinical trial sites for prospective patients.

We may not be able to initiate or continue conducting clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these clinical trials as required by the FDA or similar regulatory authorities outside the United States. Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. In particular, there may be low or slow enrollment, and the studies may enroll subjects that do not meet the inclusion criteria, requiring the erroneously enrolled subjects to be excluded and the trial population to be increased. Moreover, patients in our clinical trials, especially patients in our control groups, may be at risk for dropping out of our studies if they are not experiencing relief of their disease. A significant number of withdrawn patients would compromise the quality of a study's data.

Enrollment difficulties or delays in our clinical trials may result in increased development costs for our product candidates, or the inability to complete development of our product candidates, which would cause our value to decline, limit our ability to obtain additional financing, and materially impair our ability to generate revenues.

Data from preclinical studies and early-stage clinical trials and "top-line", initial or preliminary data from our clinical trials may not be predictive of success in later clinical trials.

The results of preclinical studies, preliminary study results, and early-stage clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials or the ultimately completed clinical trial. Preliminary and final results from such studies may not be representative of study results that are found in larger, controlled, blinded, and more long-term studies. 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. A number of companies have suffered significant setbacks in advanced clinical trials, notwithstanding promising results in earlier clinical trials. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols, and the rate of dropout among clinical trial participants.

In addition, from time to time, we may publish interim, "top-line," initial, or preliminary data from our clinical trials. Interim data from clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data becomes available. Preliminary, initial, or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data previously published. As a result, interim, "top-line", initial, and preliminary data should be viewed with caution until the final data are available. Adverse changes between preliminary, initial, "top-line" or interim data and final data could significantly harm our business prospects.

We may, in the future, conduct clinical trials for product candidates at sites outside the United States, and the FDA may not accept data from clinical trials conducted in such locations.

We may, in the future, choose to conduct one or more of our clinical trials outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of data is in either case subject to the respective conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and be performed by qualified investigators in accordance with ethical principles, such as IRB or ethics committee approval and informed consent. The trial population must also adequately represent the United States population, and the data must be applicable to the United States population and United States medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials are subject to the applicable local laws (and therefore failure to comply with such laws could result in regulatory enforcement action), acceptance of the data by the FDA will be dependent upon its determination that the clinical trials were conducted consistent with all applicable United States laws and regulations. If the FDA does not accept the data from any clinical trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates.

Failure to obtain marketing approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in jurisdictions outside the United States, we must obtain separate marketing approvals in international jurisdictions and comply with numerous and varying regulatory requirements. The approval procedures vary among countries and the time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. The clinical trials of our product candidates may not be sufficient to support an application for marketing approval outside the United States. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming.

We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. We, or any current or eventual collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not guarantee approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not guarantee approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, the failure to obtain approval in one jurisdiction may compromise our ability to obtain approval elsewhere. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

We may be subject to fines, penalties, injunctions, or other enforcement actions if we are determined to be promoting the use of our products, if approved, for unapproved or "off-label" uses, resulting in damage to our reputation and business.

We must comply with requirements concerning advertising and promotion for any product candidates for which we obtain marketing approval. Promotional communications with respect to therapeutics are subject to a variety of legal and regulatory restrictions and continuing review by the FDA, Department of Justice, Department of Health and Human Services' Office of Inspector General, state attorneys general, members of Congress, and the public. When the FDA or comparable foreign regulatory authorities issue regulatory approval for a product candidate, the regulatory approval is limited to those specific uses and indications for which a product is approved. We may not market or promote them for other indications and uses, referred to as off-label uses. We further must be able to sufficiently substantiate any claims that we make for our products, if approved, including claims comparing our products to other companies' products and must abide by the FDA's strict requirements regarding the content of promotion and advertising. While physicians may choose to prescribe products for uses that are not described in the product's labeling and for uses that differ from those tested in clinical studies and approved by the regulatory

authorities, we are prohibited from marketing and promoting the products for indications and uses that are not specifically approved by the FDA.

If we are found to have impermissibly promoted any of our product candidates, we may become subject to significant liability and government fines. The FDA and other agencies actively enforce the laws and regulations regarding product promotion, particularly those prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted a product may be subject to significant sanctions. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees of permanent injunctions under which specified promotional conduct is changed or curtailed.

In the United States, engaging in the impermissible promotion of our products, following approval, for off-label uses can also subject us to false claims and other litigation under federal and state statutes, including fraud and abuse and consumer protection laws. Such litigation can lead to civil and criminal penalties and fines, agreements with governmental authorities that materially restrict the manner in which we promote or distribute therapeutic products and do business through, for example, corporate integrity agreements, suspension or exclusion from participation in federal and state healthcare programs, suspension and debarment from government contracts, and refusal of orders under existing government contracts. These false claims statutes include the federal civil FCA, which allows any individual to bring a lawsuit against a company on behalf of the federal government ("qui tam" action) alleging submission of false or fraudulent claims, or causing others to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government decides to intervene and prevails in the lawsuit, the individual will share in the proceeds from any fines or settlement funds. If the government declines to intervene, the individual may pursue the case alone. These FCA lawsuits against sponsors of drugs and biologics have increased significantly in volume and breadth in recent years, leading to several substantial civil and criminal settlements, up to $3.0 billion, pertaining to certain sales practices and promoting off-label uses. In addition, FCA lawsuits may expose sponsors to follow-on claims by private payors based on fraudulent marketing practices. This growth in litigation has increased the risk that companies will have to defend a false claim action, and pay settlements fines or restitution, as well as criminal and civil penalties, agree to comply with burdensome reporting and compliance obligations, and be excluded from Medicare, Medicaid, or other federal and state healthcare programs. If we do not lawfully promote our approved products, if any, we may become subject to such litigation and, if we do not successfully defend against such actions, those actions may have a material adverse effect on our business, financial condition, results of operations, and prospects.

In the United States, the distribution of product samples to physicians must further comply with the requirements of the United States PDMA, and the promotion of biologic and pharmaceutical products are subject to additional FDA requirements and restrictions on promotional statements. If the FDA determines that our promotional activities violate our regulations and policies pertaining to product promotion, it could request that we modify our promotional materials or subject us to regulatory or other enforcement actions, including issuance of warning letters or untitled letters, suspension or withdrawal of an approved product from the market, requests for recalls, payment of civil fines, disgorgement of money, imposition of operating restrictions, injunctions or criminal prosecution, and other enforcement actions. These regulatory and enforcement actions could significantly harm our business, financial condition, results of operations, and prospects.

Even if our product candidates receive regulatory approval, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense.

Any product candidate for which we obtain marketing approval will be subject to extensive and ongoing requirements of and review by the FDA and other regulatory authorities, including requirements related to the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising, marketing, and promotional activities for such product. These requirements further include submissions of safety and other post-marketing information, including manufacturing deviations and reports, registration and listing requirements, the payment of annual fees, continued compliance with current GMP requirements relating to manufacturing, quality control, quality assurance, and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and GCPs, for any clinical trials that we conduct post-approval.

Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses and populations for which the product may be marketed or to the conditions of approval, including significant safety warnings, such as boxed warnings, contraindications, and precautions that are not desirable for successful commercialization. Any approved products may also be subject to a REMS that render the approved product not commercially viable or other post-market requirements, such as Phase 4 studies, or restrictions. If the FDA or comparable foreign regulatory authorities become aware of new safety information after the approval of any of our product candidates, they may, among other actions, withdraw approval, require labeling changes or establishment of a REMS or similar strategy, impose significant restrictions on a

product's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance.

We and any of our collaborators, including our contract manufacturers, could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with current GMP and other FDA regulatory requirements. Application holders must further notify the FDA, and depending on the nature of the change, obtain FDA pre-approval for product and manufacturing changes.

In addition, later discovery of previously unknown adverse events or that the product is less effective than previously thought or other problems with our products, manufacturers, or manufacturing processes, or failure to comply with regulatory requirements both before and after approval, may yield various results, including:

- restrictions on manufacturing, distribution, or marketing of such products;
- restrictions on the labeling, including restrictions on the indication or approved patient population, and required additional warnings, such as black box warnings, contraindications, and precautions;
- modifications to promotional pieces;
- issuance of corrective information;
- requirements to conduct post-marketing studies or other clinical trials;
- clinical holds or termination of clinical trials;
- requirements to establish or modify a REMS, or a comparable foreign authority may require that we establish or modify a similar strategy;
- liability for harm caused to patients or subjects;
- reputational harm;
- warning, untitled, Form 483s, or cyber letters;
- suspension of marketing or withdrawal or recall of the products from the market;
- regulatory authority issuance of safety alerts, Dear Healthcare Provider letters, press releases, or other communications containing warnings or other safety information about the product;
- refusal to approve pending applications or supplements to approved applications that we submit;
- fines, restitution, or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure or detention;
- FDA debarment, suspension and debarment from government contracts, and refusal of orders under existing government contracts, exclusion from federal healthcare programs, consent decrees, or corporate integrity agreements; or
- injunctions or the imposition of civil or criminal penalties, including imprisonment.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, or could substantially increase the costs and expenses of developing and commercializing such product, which in turn could delay or prevent us from generating significant revenues from its sale. Any of these events could further have other material and adverse effects on our operations and business and could adversely impact our stock price and could significantly harm our business, financial condition, results of operations, and prospects.

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, limit the marketability of our product candidates, or impose additional regulatory obligations on us. Changes in medical practice and standard of care may also impact the marketability of our product candidates, if approved.

We will need to obtain FDA approval of any proposed product names, and any failure or delay associated with such approval may adversely affect our business.

Any name we intend to use for our product candidates will require approval from the FDA regardless of whether we have secured a formal trademark registration from the USPTO. The FDA typically conducts a review of proposed product names, including an evaluation of the potential for confusion with other product names. The FDA may also object to a product name if it believes the name inappropriately implies medical claims or contributes to an overstatement of efficacy. If the FDA objects to any of our proposed product names, we may be required to adopt alternative names for our product candidates. If we adopt alternative names, we would lose the benefit of any existing trademark applications for such product candidate and may be required to expend significant additional resources in an effort to identify a suitable product name that would qualify under applicable trademark laws, not infringe upon the existing rights of third-parties, and be acceptable to the FDA. We may be unable to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product candidates, if approved.

Geopolitical events and conditions could adversely affect our business, financial condition and operating results.

Changes in U.S. government and other nations' administrations and their associated shifts in policy and priorities could also impact our operations and market conditions. Our business is highly sensitive to geopolitical issues, including foreign policy actions taken by governments such as tariffs, sanctions, embargoes, export and import controls, and other trade restrictions, which can affect the demand for, and our ability to sell, our products and services, cause disruptions to our supply chain, and, ultimately, could adversely affect our business. Global conflicts, including Russia's invasion of Ukraine, conflicts in the Middle East, and heightened tensions in the Pacific region, have significantly elevated global geopolitical tensions and security concerns. Economic sanctions, export controls, and other trade restrictions, for instance those that the U.S. Government and other nations implemented against Russia in light of its invasion of Ukraine or those relating to the conflict in the Middle East, could directly and indirectly result in the disruption of our business and supply chain. Although we do not have any clinical trial sites or operations in the currently affected regions, if the current conflict expands further into the region or continues, resulting heightened economic sanctions from the United States and the international community, in addition to environmental regulations, could limit our ability to procure or use certain materials or manufacturing slots for the products needed for our clinical trials, which could lead to delays in these trials.

However, portions of our clinical trials are conducted outside of the United States, such as our Phase 3 liMeliGhT clinical trial for OCU400 for the treatment of RP in Canada, and unfavorable economic conditions resulting in the weakening of the U.S. dollar would make those clinical trials more costly to operate. Significant political, trade, or regulatory developments in the jurisdictions in which we sell our products, if approved, such as those stemming from the change in U.S. federal administration, are difficult to predict and may have a material adverse effect on us. Similarly, changes in U.S. federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. For example, on February 1, 2025, the U.S. imposed a 25% tariff on imports from Canada and Mexico, which were subsequently suspended for a period of one month, and a 10% additional tariff on imports from China. Historically, tariffs have led to increased trade and political tensions. In response to tariffs, other countries have implemented retaliatory tariffs on U.S. goods. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our financial condition or results of operations.

Specifically, changes in the economic, political, legal and social conditions and policies of the Chinese government or in relations between China and the United States may materially and adversely affect our business, financial condition, results of operations, access to capital, and the market price of our common stock.

We have a co-development and commercialization agreement with CanSinoBIO with respect to the development and commercialization of our modifier gene therapy platform including OCU400, OCU410 and OCU410ST as well as manufacturing part of our inhaled mucosal vaccine platform including OCU500, and as a result, have significant operations in China. Due to our operations in China, our business, results of operations, financial condition, access to capital, market price of our common stock and prospects may be influenced to a significant degree by economic, political, legal and social conditions in China or changes in government relations between China and the United States or other governments. Furthermore, we face the risk that our business operations in China will be impacted by government regulations and/or foreign sanctions. Escalation of current geopolitical tensions may implicate China and could increase the risk of government regulations and/or foreign sanctions and imposition of export controls and import restrictions. In addition, our information technology systems may be at risk of being blocked from our world-wide operations. Ongoing human rights concerns in China may result in boycotts of our services or client requests not to use Chinese operations to support their projects.

In addition, the U.S. Congress has previously considered – and is anticipated to consider in the future – legislative proposals such as the BIOSECURE ACT that, if enacted, could negatively impact the United States funding for certain biotechnology

providers having relationships with foreign adversaries or which pose a threat to national security, including entities located in China. The potential downstream adverse impacts on entities having only commercial relationships with any impacted biotechnology providers is unknown but may include supply chain disruptions or delays.

Inadequate funding for the FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies' operations could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business

Currently, federal agencies in the US are operating under a continuing resolution that is set to expire on March 14, 2025. Without appropriation of additional funding to federal agencies, our business operations related to our product development activities for the US market could be impacted. The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, the ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

The U.S. Congress, the Trump administration, or any new administration may make substantial changes to fiscal, tax, and other federal policies that may adversely affect our business.

In 2017, The U.S. Congress and the Trump administration made substantial changes to U.S. policies, which included comprehensive corporate and individual tax reform. In addition, the Trump administration called for significant changes to U.S. trade, healthcare, immigration and government regulatory policy. With the transition to the Biden administration in early 2021, changes to U. S. policy occurred and since the start of the Trump Administration in 2025, U.S. policy changes have been implemented at a rapid pace and additional changes are likely. Changes to U.S. policy implemented by the U.S. Congress, the Trump administration or any new administration have impacted and may in the future impact, among other things, the U.S. and global economy, international trade relations, unemployment, immigration, healthcare, taxation, the U.S. regulatory environment, inflation and other areas. Although we cannot predict the impact, if any, of these changes to our business, they could adversely affect our business. Until we know what policy changes are made, whether those policy changes are challenged and subsequently upheld by the court system and how those changes impact our business and the business of our competitors over the long term, we will not know if, overall, we will benefit from them or be negatively affected by them.

Risks Related to the Commercialization of Our Product Candidates

We have no prior experience in the marketing, sale, and distribution of biotechnology products and there can be no assurance that our product candidates, if approved, will be successfully commercialized.

We have no prior experience in the marketing, sale, and distribution of biotechnology products, and there are significant risks involved in the building and managing of a commercial infrastructure. The establishment and development of commercial capabilities, including compliance plans, to market any products we may develop will be expensive and time-consuming and could delay any product launch, and we may not be able to successfully develop this capability. We will have to compete with other pharmaceutical or biotechnology companies to recruit, hire, train, manage, and retain marketing and sales personnel. Factors that may inhibit our efforts to commercialize our product candidates include:

- the inability to recruit, train, manage, and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe our product candidates;
- our inability to effectively oversee a geographically dispersed sales and marketing team;
- the costs associated with training sales and marketing personnel on legal and regulatory compliance matters and monitoring their actions;
- an inability to secure adequate coverage and reimbursement by government and private health plans;

- reduced realization on government sales from mandatory discounts, rebates and fees, and from price concessions to private health plans and pharmacy benefit managers necessitated by competition for access to managed formularies;
- the clinical indications for which the products are approved and the claims that we may make for the products;
- limitations or warnings, including distribution or use restrictions, contained in the products' approved labeling;
- any distribution and use restrictions imposed by the FDA or other foreign regulatory agencies, including those that we may agree to as part of a mandatory REMS or voluntary risk management plan;
- liability for sales or marketing personnel who fail to comply with the applicable legal and regulatory requirements;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization or engaging a contract sales organization.

Should any of the foregoing occur, we may not be successful in commercializing any product candidates for which we receive marketing approval.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to conducting marketing and sales activities in international jurisdictions and entering into international business relationships, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- the need to seek additional patent approvals, licenses to patents held by third parties, and/or face claims of infringing third-party patent rights;
- tariffs (including tariffs that have been or may in the future be imposed by the United States or other countries), trade protection measures, import or export licensing requirements, trade embargoes, sanctions (including those administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury), other trade barriers (including further legislation or actions taken by the United States or other countries that restrict trade), and protectionist or retaliatory measures taken by the United States or other countries;
- economic weakness, including inflation or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration, and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the FCPA, the U.K. Bribery Act 2010 (the "Bribery Act"), or other comparable foreign regulations;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including pandemics or other outbreaks of infectious disease, earthquakes, typhoons, floods, and fires.

These and other risks associated with international operations may materially adversely affect our ability to attain or maintain profitable operations.

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

We currently do not have a commercial infrastructure for the marketing, sale, and distribution of biotechnology products. If approved, in order to commercialize our products, we must build our marketing, sales, and distribution capabilities or make arrangements with third parties to perform these services. If we do not establish sales, marketing, and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates for which we receive marketing approval.

Subject to regulatory approval of any of our product candidates, we may build a commercial team of specialty sales and marketing representatives in support of our product candidates that we develop in the United States or other foreign countries, if approved, as well as distribution capabilities. There are risks involved with us establishing our own sales, marketing, and distribution capabilities. Recruiting and training a sales force is expensive and time-consuming, particularly to the extent that we seek to commercialize any product, if approved, for an indication, such as dAMD, that has a large patient population. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations to recruit, hire, train, and retain marketing and sales personnel. Further, we may underestimate the size of the sales force required for a successful product launch and may need to expand our sales force earlier and at a higher cost than we anticipate. If the commercial launch of our product candidates, if approved, for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may also or alternatively decide to collaborate with a third-party or contract sales organization to commercialize any approved product candidates, in which event, our ability to generate product revenues may be limited. Our product revenues and our profitability, if any, under any third-party collaboration, distribution, or other marketing arrangements are likely to be lower than if we were to market, sell, and distribute the applicable product candidate, if approved, entirely ourselves. We may not be successful in entering into arrangements with third parties to sell, market, and distribute our product candidates, if approved, or may be unable to do so on terms that are favorable to us. In addition, we would have less control over the sales efforts of any other third parties involved in our commercialization efforts and any of them may fail to devote the necessary resources and attention to sell and market our product candidates, if approved, effectively. We could also be held liable if such third parties failed to comply with applicable legal or regulatory requirements.

In the event we are unable to develop a team of marketing and sales representatives or to establish an effective third-party contractual relationship for such services, we may not be able to commercialize our product candidates, if approved, which would limit our ability to generate product revenues. Even if we are able to effectively hire a sales force and develop a marketing and sales infrastructure, our sales force and marketing teams may not be successful in commercializing our product candidates, if approved.

We face significant competition from other pharmaceutical and biotechnology companies, academic institutions, government agencies, and other research organizations. Our operating results will suffer if we fail to compete effectively.

The biotechnology industry is characterized by rapidly advancing technologies as well as a strong emphasis on intellectual property leading to a highly competitive environment for the development and commercialization of therapeutic products, regenerative medicines, and vaccines. We face competition with respect to our current product candidates and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future. We face competition from many different sources, including from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

The development and commercialization of gene therapies is highly competitive. We are aware of several companies focusing on gene therapies for various ophthalmic indications including Applied Genetic Technologies Corporation, as acquired by Syncona Limited, Astellas Pharma Inc., MeiraGTx Holdings plc in partnership with Janssen Pharmaceuticals, Inc., Nanoscope Therapeutics Inc., REGENXBIO Inc., Novartis AG, Roche AG, Kiora Pharmaceuticals, Inc., Genentech, Inc. in partnership with Lineage Cell Therapeutics, Inc., and Spark Therapeutics, Inc. Luxturna, the product developed by Spark Therapeutics, Inc. and marketed by Roche AG, is currently the only gene therapy approved to treat IRDs in the United States which addresses only mutations in the *RPE65* gene. The mutation associated with the *RPE65* gene represents just one of more than 125 mutated genes linked to RP and LCA.

The regenerative medicine sector is characterized by innovative science, rapidly advancing technologies, and a strong emphasis on proprietary products. The competitive landscape in the field of articular cartilage repair in the United States is emerging and has stimulated a substantial amount of interest from companies developing tissue repair solutions. Products that may compete with our NeoCart product candidate include Vericel Corporation's MACI, the only FDA-approved ACI product in the United States, and Aesculap Biologics, LLC's NOVOCART 3D, which is currently enrolling subjects in their Phase 3 clinical trial.

We face, and will continue to face, intense competition from companies as well as institutions that are pursuing or have commercialized vaccines that would compete with our inhaled mucosal vaccine platform, if commercialized. The competitive landscape of COVID-19 vaccines includes competitors such as Pfizer Inc./BioNTech SE, Moderna, Inc., AstraZeneca PLC, Novavax, Inc., Sinovac Biotech Ltd., Gamaleya Research Institute of Epidemiology and Microbiology, and Center for Genetic Engineering and Biotechnology. Each of the aforementioned vaccines have been authorized or approved in at least one country within the Ocugen Mucosal Vaccine Territory and are intramuscular vaccines. CanSinoBIO's Convidecia Air, an intranasal vaccine targeting COVID-19, has been approved in China. Other competitors for our novel inhaled mucosal vaccine platform include CyanVac LLC, Meissa Vaccines, Inc., Codagenix, Inc., Intravacc B.V., McMaster University, and Tetherex Pharmaceuticals Corporation. Companies such as Pfizer Inc./BioNTech SE, Moderna, Inc., CureVac N.V in partnership with GSK plc, Vivaldi Biosciences Inc., and Novavax, Inc. are also in the process of developing a combination vaccine that will protect against COVID-19 and the seasonal flu. Vivaldi Biosciences Inc. is also currently undergoing clinical trials for their intranasal vaccine for the seasonal flu.

The development and commercialization of biologic products is highly competitive as well. Companies that may compete with our OCU200 product candidate include Roche AG, Regeneron Pharmaceuticals, Inc., AsclepiX Therapeutics, Inc., Outlook Therapeutics, Inc., Novartis AG, Oxurion NV, Unity Biotechnology, Inc., Opthea Limited, and 4D Molecular Therapeutics, Inc. Roche AG, Regeneron Pharmaceuticals, Inc., and Novartis AG have marketed anti-VEGF products.

Our commercial opportunities 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 and for which we receive approval. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market. They may obtain patent protection or other intellectual property rights that allow them to develop and commercialize their products before us and could limit our ability to develop or commercialize our product candidates.

In addition, our ability to compete may be affected in many cases by insurers or other third-party payors' coverage decisions, particularly Medicare, seeking to encourage the use of generic or biosimilar products. Many of the products that will compete with our product candidates, if approved, are available on a generic basis, and our product candidates may not demonstrate sufficient additional clinical benefits to clinicians, patients, or payors to justify a higher price compared to generic products. Additional competing products are expected to become available on a generic basis over the coming years.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If our product candidates for which we receive approval do not achieve broad market acceptance, the revenues that we generate from their sales will be limited.

Even if our product candidates are approved by the appropriate regulatory authorities for marketing and sale, they may not gain acceptance among physicians, patients, third-party payors, and others in the medical community. Physicians are often reluctant to switch their patients and patients may be reluctant to switch from existing therapies even when new and potentially more effective or safer treatments enter the market. We have never commercialized a product candidate for any indication, and efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. With respect to our product candidates being developed based on our modifier gene therapy platform, market acceptance may also be constrained by ethical, social, and legal concerns about gene therapy and genetic research, which could result in additional regulations restricting or prohibiting the products and processes we may use. The novelty of the technology and any negative publicity surrounding adverse events associated with gene therapy may also

prevent the medical community, patients, and third-party payors from accepting gene therapy products in general, and our product candidates in particular, as medically useful, cost-effective, and safe.

Market acceptance of our product candidates by the medical community, patients, and third-party payors will depend on a number of factors, some of which are beyond our control. If any product candidates for which we obtain regulatory approval does not gain an adequate level of market acceptance, it may not generate significant product revenues or become profitable.

The degree of market acceptance of any of our product candidates will depend on a number of factors, including:

- the efficacy of our product candidates;
- the prevalence and severity of adverse events associated with such product candidates;
- the clinical indications for which the product candidates are approved and the approved claims that we may make for the products;
- limitations or warnings contained in the product's FDA-approved labeling, including potential limitations or warnings for such product candidates that may be more restrictive than other competitive products;
- changes in the standard of care for the targeted indications for such product candidates, which could reduce the marketing impact of any claims that we could make following FDA approval, if obtained;
- the relative convenience and ease of administration of such product candidates;
- cost of treatment versus economic and clinical benefit in relation to alternative treatments or therapies;
- the availability of third-party formulary coverage and adequate coverage or reimbursement by third parties, such as insurance companies and other healthcare payors, and by government healthcare programs, including Medicaid and particularly by Medicare in light of the prevalence of retinal diseases in persons over age 55;
- the price concessions required by third party payors to obtain coverage;
- the extent and strength of our manufacturing, marketing, and distribution of such product candidates;
- distribution and use restrictions imposed by the FDA with respect to such product candidates or to which we agree as part of a REMS or voluntary risk management plan;
- the extent of availability of generic or biosimilar versions of any products that compete with any of our product candidates and the extent to which they are offered at a substantially lower price than we expect to offer for our product candidates, if approved;
- adverse publicity about the product or favorable publicity about competitive products; and
- potential product liability claims.

If the market opportunities for our product candidates are smaller than we believe, our revenue may be adversely affected, and our business may suffer.

The potential market opportunities for our product candidates are difficult to precisely estimate. Our estimates of the potential market opportunities are predicated on many assumptions, which may include industry knowledge and publications, third-party research reports, and other surveys, some of which we may have commissioned. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third-party research, surveys, and studies are reliable, we have not independently verified such data. In addition, while we believe that our internal assumptions are reasonable, these assumptions involve the exercise of significant judgment on the part of our management, are inherently uncertain, and the reasonableness of these assumptions has not been assessed by an independent source. If any assumptions prove to be inaccurate, the actual markets for our product candidates could be smaller than our estimates of the potential market opportunities, and as a result, our product revenue may be limited, and it may be more difficult for us to achieve or maintain profitability.

If third-party payors do not reimburse patients for our products candidates, if approved, or if reimbursement levels are set too low for us to sell our product candidates at a profit, our ability to successfully commercialize our product candidates, if approved, and our results of operations will be harmed.

Our ability to successfully commercialize our product candidates, if approved, will depend in part on the extent to which coverage and adequate reimbursement for our product candidates will be available in a timely manner from third-party payors,

including governmental healthcare programs such as Medicare and Medicaid, commercial health insurers, and managed care organizations. This is particularly true with respect to OCU200, our novel biologic product candidate, in the case of Wet AMD, which is most prevalent in persons over age 55. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels. Reimbursement decisions by particular third-party payors depend upon a number of factors, including each third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- appropriate and medically necessary for the specific condition or disease;
- cost effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement approval for our product candidates from government authorities or other third-party payors may be a time consuming and costly process that could require us to provide supporting scientific, clinical, and cost-effectiveness data, including expensive pharmacoeconomic studies beyond the data required to obtain marketing approval, for the use of each product candidate to each government authority or other third-party payor. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement.

Third-party payors may deny reimbursement for covered products if they determine that a medical product was not used in accordance with cost-effective diagnosis methods, as determined by the third-party payor, or was used for an unapproved indication. Third-party payors also may refuse to reimburse for procedures and devices deemed to be experimental. Third-party payors may also limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication.

Increasingly, third-party payors are also requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. These third-party payors could also impose price controls and other conditions that must be met by patients prior to providing coverage for use of our product candidates, if approved. For example, insurers may establish a "step-edit" system that requires a patient to first use a lower price alternative product prior to becoming eligible for reimbursement of a higher price product.

Third-party payors also attempt to contain healthcare costs by limiting both coverage and the level of reimbursement for medical products and services. The process for determining whether a payor will provide coverage for a product may be separate from the process of setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Levels of reimbursement may also decrease in the future, and legislation, regulation, or reimbursement policies of third-party payors may adversely affect the demand for and reimbursement available for our product candidates, which in turn, could negatively impact pricing. If patients are not adequately reimbursed for our product candidates, if approved, they may reduce or discontinue purchases of our product candidates, which would result in a significant shortfall in achieving revenue expectations and negatively impact our business, prospects, and financial condition. For example, the IRA contains substantial drug pricing reforms, including the establishment of a drug price negotiation program within the United States Department of Health and Human Services that would require manufacturers to charge a negotiated "maximum fair price" for certain selected drugs administered or prescribed to Medicare beneficiaries or pay an excise tax for noncompliance; the establishment of rebate payment requirements on manufacturers of certain drugs payable under Medicare Parts B and D to penalize price increases that outpace inflation; and a requirement that manufacturers to provide discounts on Part D drugs. Substantial penalties can be assessed for noncompliance with the drug pricing provisions in the IRA. The IRA could have the effect of reducing the prices we can charge and reimbursement we receive for our products, if approved, thereby reducing our profitability, and could have a material adverse effect on our financial condition, results of operations, and growth prospects. The IRA's drug price negotiation provisions are subject to ongoing constitutional challenges, and the effect of IRA on our business and the biotechnology industry in general is not yet known.

As an organization, we have limited experience in the development, manufacturing, distribution, or commercialization of a vaccine candidate.

We have limited experience in the development of vaccine candidates and have never undertaken the manufacturing, distribution, or commercialization of a vaccine candidate, and we may be unable to obtain regulatory authorization or approval. Additionally, development of an effective vaccine candidate depends on the success of our and our partner’s manufacturing capabilities. In addition, the development and manufacture of inhaled mucosal vaccines may prove to be more complex than the development and manufacture of traditional vaccines. Further, we may be unable to effectively create a supply chain for our vaccines that will adequately support demand. We may also face challenges with sourcing a sufficient amount of raw materials

to support the demand for a vaccine. While we are currently collaborating with NIAID for early clinical studies for the OCU500 program, there can also be no assurance that we will be able to obtain the required funding from government agencies or strategic partners for further development of OCU500 or for development of our vaccine candidates, OCU510 and OCU520.

Due to the end of the COVID-19 public health emergency and decline in vaccination rates, the demand for any COVID-19 vaccine product candidate we develop may decrease significantly.

On May 11, 2023, the United States Department of Health and Human Services announced the COVID-19 public health emergency ended. In addition, the demand for COVID-19 vaccines is becoming more endemic and seasonal. As other companies continue to develop, receive regulatory approval for and commercialize their own COVID-19 vaccines and as demand for such vaccines declines, demand for our COVID-19 product candidates OCU500 and OCU520 may be diminished.

Risks Related to Our Dependence on Third Parties

We rely, and expect to continue to rely, on third parties to conduct, supervise, and monitor our preclinical studies and clinical trials we may initiate, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such preclinical studies or clinical trials or failing to comply with regulatory requirements.

We rely on third parties, study sites, and others to conduct, supervise, and monitor our preclinical and clinical trials for our product candidates. We expect to continue to rely on third parties, such as CDMOs, clinical data management organizations, medical and scientific institutions, and clinical and preclinical investigators to conduct our preclinical studies and clinical trials. We often have to negotiate budgets and contracts with such third parties, and if we are unsuccessful or if the negotiations take longer than anticipated, this could result in delays to our development timelines and increased costs.

While we have, or expect to have, agreements governing the activities of such third parties, we will have limited influence and control over their actual performance and activities. Third-party service providers are not our employees, and except for remedies available to us under agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our preclinical studies or clinical trials. Nevertheless, we will be responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol and legal, regulatory, and scientific standards, and our reliance on third parties will not relieve us of our regulatory responsibilities. For example, we will remain responsible for ensuring that each of our trials is conducted in accordance with the general investigational plan and protocols for the trial. We must also ensure that our preclinical studies are conducted in accordance with GLP and under current GMP conditions, 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. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites, and IRBs.

Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violate federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

If these third parties upon which we depend do not successfully carry out their contractual duties, meet expected deadlines, conduct our preclinical studies or any clinical trials in accordance with regulatory requirements or our stated protocols, 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, our CDMOs, or other third-party collaborators may be subject to regulatory enforcement or other legal actions;
- the data generated in our preclinical studies or clinical trials may be deemed unreliable and our such studies and clinical trials may need to be repeated, extended, delayed, or terminated;
- we may need to identify new CDMOs with which to partner for the supply of our product candidates;
- we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates; or
- we may not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates, if approved.

We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our preclinical studies or clinical trials will comply with the applicable regulatory requirements. To the extent we are unable to successfully identify and manage the performance of third-party service providers in the future, our business may be materially

and adversely affected. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If we need to identify and retain alternative CDMOs for any reason during our product development programs, the technical skills required to manufacture our product candidates may be unique or proprietary to the original CDMO and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change CDMOs for any reason, we will be required to verify that the new CDMO maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new CDMO could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a CDMO may possess technology related to the manufacture of our product candidate that such CDMO owns independently. This would increase our reliance on such CDMO or require us to obtain a license from such CDMO in order to have another CDMO manufacture our product candidates. In addition, changes in manufacturers often involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacturer. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

Our reliance on third parties for clinical trials will entail additional risks. Our third-party service providers may have relationships with other entities, some of which may be our competitors, for whom they may also be conducting clinical trials or other therapeutic development activities that could harm our competitive position. In addition, we will be required to report certain financial interests of our third-party investigators if these relationships exceed certain financial thresholds or meet other criteria. The FDA or comparable foreign regulatory authorities may question the integrity of the data from those clinical trials conducted by investigators who may have conflicts of interest. Lastly, we are required to register certain clinical trials and post the results of certain completed clinical trials on a government-sponsored database, *clinicaltrials.gov*, within specified timeframes. Failure to do so can result in enforcement actions and adverse publicity.

Agreements with third parties conducting or otherwise assisting with our clinical or preclinical studies might terminate for a variety of reasons, including a failure to perform by the third parties. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative providers or do so on commercially reasonable terms. Switching or adding additional third parties involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, if we need to enter into alternative arrangements, it could delay our product development activities and adversely affect our business. Though we intend to carefully manage our relationships with third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, prospects, and results of operations.

We will also rely on other third parties to store and distribute our product candidates for preclinical purposes or clinical trials that we conduct. Any performance failure on the part of our distributors could delay development, marketing approval, or commercialization of our product candidates, if approved, producing additional losses and depriving us of potential product revenue.

If we encounter difficulties in negotiating commercial manufacturing and supply agreements with third-party manufacturers and suppliers of our product candidates or any product components, our ability to commercialize our product candidates, if approved, would be impaired.

We have entered into a strategic partnership with CanSinoBIO to manufacture our modifier gene therapy pipeline product candidates. Under this agreement, CanSinoBIO is responsible for the CMC development and manufacture of clinical supplies for OCU400, OCU410 and OCU410ST. The agreement also provides commercialization rights to CanSinoBIO in Greater China. This agreement may be adversely affected if the United States government were to impose restrictions related to goods manufactured in or imported from China.

We expect to rely on our qualified suppliers and other third parties to manufacture clinical supplies of our product candidates and commercial supplies of our products, if and when approved for marketing by applicable regulatory authorities, as well as for packaging, serialization, storage, distribution, and other production logistics. We, however, may not succeed in our efforts to establish manufacturing relationships or other alternative arrangements for any of our product candidates, components, and programs, or may be unable to do so on commercially favorable terms. If we are unable to enter into such agreements on commercially favorable terms, our future profit margins would be adversely affected and our ability to commercialize any

products that receive marketing approval on a timely and competitive basis would be impaired. As a result, our business, financial condition, and results of operations would be materially adversely affected.

If the manufacturers upon whom we rely on fail to produce our product candidates or product components pursuant to the terms of contractual arrangements with us or fail to comply with stringent regulations applicable to biotechnology manufacturers, we may face delays in the development and commercialization of, or be unable to meet demand for, our product candidates, if approved, and may lose potential revenues.

We rely on third-party contract manufacturers to manufacture some of our pre-clinical product candidate supplies and some of our clinical trial product supplies and, if approved, will rely on third-party contract manufacturers to manufacture some of our commercial product supplies, including all of our drug substance, vialing, labeling, and packaging. We do not own manufacturing facilities for producing any clinical trial or commercial product supplies. There can be no assurance that our preclinical, clinical development, and, if approved, commercial product supplies will not be limited or interrupted, including as a result of impacts of current macroeconomic and geopolitical events, increasing rates of inflation, rising interest rates, or that our product supplies will be of satisfactory quality or continue to be available at acceptable prices.

As with the third parties on which we rely or expect to rely for our preclinical activities and clinical trials, we have agreements governing the activities of our manufacturers but have limited influence and control over their actual performance and activities. Our third-party manufacturers are not our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our manufacturing requirements. If these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements, and if there are disagreements between us and such parties, clinical development or marketing approval of our product candidates could be delayed.

The manufacture of biotechnology 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 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.

In addition, all manufacturers of our product candidates and therapeutic substances must comply with current GMP requirements enforced by the FDA that are applicable to both finished products and their active components used for both clinical and commercial supply. The FDA enforces these requirements through its facilities inspection program. Our manufacturers must be approved by the FDA pursuant to inspections that will be conducted after we submit our marketing applications to the agency. Our manufacturers will also be subject to continuing FDA and other regulatory authority inspections should we receive marketing approval. Further, we, in cooperation with our contract manufacturers, must supply all necessary CMC documentation to the FDA in support of a marketing application on a timely basis.

The current GMP requirements include quality control, quality assurance, and the maintenance of records and documentation. Manufacturers of our product candidates, therapeutic substances, and the active pharmaceutical ingredients necessary to produce our product candidates may be unable to comply with our specifications, current GMP 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 our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, they 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 that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any resulting delays in obtaining products, if approved, 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.

While we are ultimately responsible for the manufacture of our product candidates, other than through our contractual arrangements, we have little control over our manufacturers' compliance with these regulations and standards. A failure to comply with the applicable regulatory requirements may result in regulatory enforcement actions against our manufacturers or us, including fines and civil and criminal penalties, including imprisonment, suspension or restrictions of production, injunctions, delays, withdrawal or denial of product approval or supplements to approved products, clinical holds or termination

of clinical studies, warning or untitled letters, Form 483s, regulatory authority communications warning the public about safety issues with the product, refusal to permit the import or export of the products, product seizure, detention, or recall, operating restrictions, suits under the civil FCA, corporate integrity agreements, or consent decrees. Depending on the severity of any potential regulatory action, our clinical or commercial supply could be interrupted or limited, which could have a material adverse effect on our business.

Any problems or delays we experience in preparing for commercial-scale manufacturing of a product candidate or component, including manufacturing validation, may result in a delay in FDA approval or commercial launch, if approved, of the product candidate or may impair our ability to manufacture commercial quantities or such quantities at an acceptable cost, which could result in the delay, prevention, or impairment of commercialization of our product candidates and could adversely affect our business. The risks associated with any problems or delays may be greater should the United States government impose restrictions relating to goods manufactured in or imported from China.

We or our third-party manufacturers may also encounter shortages in the materials necessary to produce our product candidates in the quantities needed for our clinical trials or, if our product candidates are approved, in sufficient quantities for commercialization.

We or our third-party manufacturers may also encounter shortages in the raw materials, therapeutic substances, or active pharmaceutical ingredients necessary to produce our product candidates in the quantities needed for our clinical trials or, if our product candidates are approved, in sufficient quantities for commercialization or to meet an increase in demand. Such shortages may occur for a variety of reasons, including capacity constraints, delays or disruptions in the market, and shortages caused by the purchase of such materials by our competitors or others. We or our third-party manufacturers' failure to obtain the raw materials, therapeutic substances, or active pharmaceutical ingredients necessary to manufacture sufficient quantities of our product candidates may cause the manufacturers to fail to deliver the required commercial quantities of our product candidates on a timely basis and at commercially reasonable prices. If such failure occurs, we would likely be unable to meet the demand for our products, if approved, and we would lose potential revenues.

The number of available, qualified third-party manufactures is limited, and if we are compelled to locate an alternative manufacturing partner, our product development activities and commercialization could be delayed and additional expense would be incurred.

There are a limited number of manufacturers that operate under current GMP regulations, that are both capable of manufacturing for us and willing to do so, and therefore our product candidates may compete with other products and product candidates for access to manufacturing facilities. Moreover, because our product candidates must be manufactured under sterile conditions, the number of manufacturers who can meet this requirement is even more limited. If our existing third-party manufacturers, or the third parties that we engage in the future to manufacture a product, if approved, or component for commercial sale or for any clinical trials we expect to initiate in the future should cease to continue to do so for any reason (including the termination of our agreements with such manufacturers, which can occur for a variety of reasons, or the bankruptcy of such manufacturers), it would be difficult to obtain a suitable alternative manufacturer. We would likely experience delays in obtaining sufficient quantities of our product candidates for us to meet commercial demand or to advance our clinical trials while we identify and qualify replacement suppliers. Any change in our manufacturers could be costly because the commercial terms of any new arrangement could be less favorable and because the expenses relating to the transfer of necessary technology and processes could be significant.

If the FDA or a comparable foreign regulatory authority inspects the facilities for the manufacture of our product candidates and finds that they are not in compliance with current GMP regulations now or in the future, we may need to find alternative manufacturing facilities. 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. Any such developments would significantly impact our ability to develop, obtain, and maintain regulatory authorization or approval for or market our product candidates, if approved.

The number of available third-party facilities may also be further limited by natural disasters, such as pandemics, floods, fire, or such facilities could face manufacturing issues, such as contamination or regulatory findings following a regulatory inspection of such facility. In such instances, an appropriate replacement third-party relationship may not be readily available to us or on acceptable terms, which would cause additional delays and increased expense and may have a material adverse effect on our business.

We may seek to collaborate with third parties for the development or commercialization of our product candidates. We may not be successful in establishing or maintaining collaborative relationships, any of which could adversely affect our ability to develop and commercialize our product candidates.

We are party to an agreement with CanSinoBIO for the development and commercialization of our modifier gene therapy platform. In the future, we may seek to enter into additional collaboration arrangements with pharmaceutical or biotechnology companies for the development or commercialization of other product candidates. We may utilize a variety of types of collaboration, distribution, and other marketing arrangements with third parties to develop and commercialize our product candidates, both inside and outside the United States. In particular, we may enter into arrangements with third parties to perform certain services in the United States or other countries if we do not establish our own sales, marketing, and distribution capabilities in such countries, or if we determine that such third-party arrangements are otherwise beneficial. We may also consider potential collaborative partnership opportunities for sales, marketing, distribution, development, or licensing or broader collaboration arrangements, including with mid-size and large pharmaceutical companies, regional and national pharmaceutical companies, and biotechnology companies.

The success of our current and future collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to collaboration arrangements. Accordingly, with respect to any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend in part on our collaborator's abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Moreover, disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercialization of the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Moreover, collaborations with pharmaceutical companies and other third parties are often terminated or allowed to expire. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Our current and future collaborations may pose a number of additional risks, including the following:

- collaborators may not pursue development of product candidates and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could fail to make timely regulatory submissions for a product candidate;
- collaborators may not comply with all applicable regulatory requirements or may fail to report safety data in accordance with all applicable regulatory requirements, which could subject them or us to regulatory enforcement actions;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product candidate or product;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation, or the preferred course of development, might cause delays or termination of the research, development, or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time consuming and expensive;

- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties or fail to maintain intellectual property rights which they license to us, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of our product candidates in the most efficient manner, or at all. If any collaborations do not result in the successful development and commercialization of our product candidates or if one of our collaborators subsequently terminates our agreement with us, we may not receive any future research funding, milestone, or royalty payments under the collaboration, as applicable. If we do not receive the funding we expect under the agreements, our development of our product candidates could be delayed, and we may need additional resources to develop our product candidates and our product platform. All of the risks relating to product development, regulatory approval, and commercialization described in this report also apply to the activities of our collaborators.

Additionally, if any collaborator of ours is involved in a business combination, the collaborator might de-emphasize or terminate development or commercialization of any product candidate licensed to them by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our reputation in the business and financial communities could be adversely affected.

Should we desire to pursue a collaboration agreement but are not able to establish collaborations, we may have to alter our development and commercialization plans and our business could be adversely affected.

For some of our product candidates, we may decide to collaborate with pharmaceutical or biotechnology companies for the development and potential commercialization of those product candidates. We face significant competition in seeking appropriate collaborators and whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, if approved, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. Should we desire to pursue a collaboration agreement but are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay our development program or one or more of our other development programs, delay our potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our product platform and our business may be materially and adversely affected.

Risks Related to Legal and Compliance Matters

We are currently, and may in the future be, subject to securities litigation, which is expensive and could divert management attention.

We are currently and have been subject to numerous securities class action lawsuits and stockholder derivative claims. See the section of this Annual Report for the fiscal year ended December 31, 2024 entitled "Legal Proceedings."

The complaints seek unspecified damages, interest, attorneys' fees, and other costs. We believe that these lawsuits are without merit and intend to vigorously defend against them. At this time, no assessment can be made as to their likely outcome or whether the outcome will be material to us. We may also become subject to additional securities class action lawsuits in the future. This risk is especially relevant for us because life sciences companies have experienced significant stock price volatility in recent years.

The cost of defending against these types of claims against us or the ultimate resolution of such claims, whether by settlement or adverse court decision, may divert management's attention and harm our business. Further, potential claimants may be encouraged to bring lawsuits based on a settlement from us or adverse court decisions against us. We cannot currently assess the likely outcome of such lawsuits, but the commencement and/or resolution of such lawsuits (particularly if the outcome were negative), could have a material adverse effect on our reputation, results of operations, financial condition, and cash flows. They could also cause a decline in the market price of our common stock.

If we fail to comply with federal and state healthcare laws, including fraud, abuse, and health and other information privacy and security laws, we could face substantial penalties and our business, financial condition, results of operations, and prospects could be adversely affected.

As a biotechnology company, we are subject to many federal and state healthcare laws, such as the federal Anti-Kickback Statute, the federal civil and criminal FCA, the civil monetary penalties statute, the Medicaid Drug Rebate statute and other price reporting requirements, the VHCA, HIPAA, the FCPA, the ACA, and similar state laws. We may also be subject to laws regarding transparency and patient privacy. Even though we do not and will not control referrals of healthcare services or bills directly to Medicare, Medicaid, or other third-party payors, certain federal and state healthcare laws, and regulations pertaining to fraud and abuse, reimbursement programs, government procurement, and patients' rights are and will be applicable to our business.

It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations, or case law involving applicable fraud, abuse, or other healthcare laws and regulations. If we or our operations are found to be in violation of any federal or state healthcare law, or any other governmental laws or regulations that applies to us, we may be subject to penalties, including civil, criminal, and administrative penalties, damages, fines, imprisonment, disgorgement, suspension and debarment from government contracts, and refusal of orders under existing government contracts, exclusion from participation in the United States federal or state health care programs, corporate integrity agreements, and the curtailment or restructuring of our operations, any of which could materially adversely affect our ability to operate our business and our financial results. Although an effective compliance program can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, reimbursement, and fraud laws may prove costly. Any action against us for the violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business.

Healthcare legislative or regulatory reform measures may have a negative impact on our business and results of operations.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system. The United States government, state legislatures, and foreign governments also have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement, and requirements for substitution of generic products for branded prescription products. In recent years, Congress has considered reductions in Medicare reimbursement levels for products administered by physicians.

CMS, the agency that administers the Medicare and Medicaid programs, also has authority to revise reimbursement rates and to implement coverage restrictions for some products. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products. While Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payers.

The ACA substantially changed the way healthcare is financed by both governmental and private insurers and significantly impacts the pharmaceutical industry. The ACA was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers, and impose additional health policy reforms. There have been significant ongoing administrative, executive, and legislative efforts to modify or eliminate the ACA. The ACA has also been subject to challenges in the courts.

Other legislative changes have been proposed and adopted since the passage of the ACA. For example, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to healthcare providers of up to 2.0% per fiscal year, which in connection with subsequent legislation are extended to 2032 unless additional Congressional action is taken. Further, the American Taxpayer Relief Act of 2012 among other things, reduced Medicare payments to several types of

providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further changes to and under the ACA remain possible, but it is unknown what form any such changes or any law proposed to replace or revise the ACA would take, and how or whether it may affect our business in the future. We expect that changes to the ACA, the Medicare and Medicaid programs, changes allowing the federal government to directly negotiate prices and changes stemming from other healthcare reform measures, especially with regard to healthcare access, financing, or other legislation in individual states, could have a material adverse effect on the healthcare industry.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that additional federal, state, and foreign healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in limited coverage and reimbursement, and reduced demand for our products, once approved, or additional pricing pressures.

Existing regulatory policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates.

The United States Supreme Court's June 2024 decision in *Loper Bright Enterprises v. Raimondo* overturned the longstanding *Chevron* doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The *Loper* decision could result in additional legal challenges to regulations and guidance issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the *Loper* decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, our business could be materially harmed.

Our employees, independent contractors, consultants, commercial partners, principal investigators, or CDMOs may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees, independent contractors, consultants, commercial partners, manufacturers, investigators, or CDMOs could include intentional, reckless, negligent, or unintentional failures to (i) comply with FDA regulations or other similar regulatory requirements, (ii) comply with manufacturing standards, including current GMP requirements, (iii) comply with applicable fraud and abuse laws, (iv) comply with federal and state data privacy, security, fraud and abuse, and other healthcare laws and regulations in the United States and abroad, (v) provide accurate information to the FDA, (vi) properly calculate pricing information required by federal programs, (vii) comply with federal procurement rules or contract terms, (viii) report financial information or data accurately, or (ix) disclose unauthorized activities to us. This misconduct could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation.

It is not always possible to identify and deter this type of misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations.

If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid, and other federal healthcare programs, individual imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws, and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, or be precluded from developing, manufacturing, and selling certain products outside the United States, which could adversely affect our business, results of operations, and financial condition.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with anti-corruption laws, including the Bribery Act, the FCPA, and other anti-corruption laws that apply to countries where we do business and may do business in the future. The Bribery Act, FCPA, and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed, or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage.

Compliance with the FCPA, in particular, is expensive and difficult, particularly in countries in which corruption is a recognized problem. The FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA, or local anti-corruption laws. We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom, the United States, Canada, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements, and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, various laws, regulations, and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-United States nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

If we are not in compliance with the Bribery Act, the FCPA, and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations, and liquidity. The SEC also may suspend or bar issuers from trading securities on the United States exchanges for violations of the FCPA's accounting provisions. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control laws by U.K., the United States, or other authorities could also have an adverse impact on our reputation, our business, results of operations, and financial condition.

Risks Related to Our Intellectual Property

As with all patent prosecution, there is no guarantee of obtaining and maintaining patent protection for our technology and product candidates. In addition, there is no guarantee that the scope of the patent protection obtained is sufficiently broad or enforceable, to prevent our competitors from developing and commercializing technology and products similar or identical to ours. Furthermore, our ability to successfully commercialize our technology and product candidates may be impaired due to circumstances that may be out of our control.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries, with respect to our proprietary technology and product candidates. We have sought to protect our proprietary position by filing in the United States and in certain foreign jurisdictions, patent applications related to our novel technologies and product candidates.

The patent prosecution process is expensive and time-consuming, and we may not have filed, maintained, or prosecuted and may not be able to file, maintain, and prosecute all necessary or desirable patents or patent applications at a reasonable cost or in a timely manner. In some cases, we may not be able to or it may be difficult to timely identify patentable aspects of our research and development output to obtain patent protection.

The patent position of pharmaceutical and biotechnology companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are uncertain. Our pending and future patent applications may fail to result in issued patents in the United States or in other foreign countries which may impact protection of our technology or product candidates, or which may effectively prevent others from commercializing competitive technologies and products. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and the standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, unlike patent law in the United States, European patent law precludes the patentability of methods of treatment of the human body and imposes substantial restrictions on the scope of claims it will grant. In addition, unlike the United States the European Patent Office typically limits the claims to those commensurate in scope with specifically disclosed embodiments. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain whether we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors 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 we may not know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. 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. Moreover, changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection for our proprietary technology and product candidates, prevent competitors from competing with us, or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. In some instances, we may need to license additional patents and trade secrets to commercialize our product candidates in certain territories.

The issuance of a patent is not conclusive as to our inventorship, ownership, scope, validity, or enforceability, and our owned and licensed 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, in whole or in part, 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 patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

In 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes a number of significant changes to the United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The USPTO developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, became effective in 2013. The first to file provisions limit the rights of an inventor to patent an invention if the inventor is not the first to file an application for patenting that invention. The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, which could have a material adverse effect on our business, financial condition, results of operations, and prospects. For example, the Leahy-Smith Act created a new administrative tribunal known as the Patent Trial and Appeals Board ("PTAB"), that provides a venue for any third parties to challenge the validity of patents at a cost that is significantly lower than district court litigation and on timelines that are much faster. Although it is not clear what, if any, long term impact the PTAB proceedings will have on the operation of our business, the outcome of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many United States patents. The availability of the PTAB as a lower-cost, faster, and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own patents will be challenged, thereby increasing the uncertainties and costs of maintaining, defending, and enforcing them.

If we are not able to obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, our business may be materially harmed.

Depending upon the timing, duration, and specifics of FDA marketing approval of our product candidates, one of the United States patents covering each of such product candidates or the use thereof may be eligible for up to five years of patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product to account for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it, or a method for manufacturing it may be extended. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened. In such cases, our competitors will likely obtain approval of competing products sooner than we might have anticipated, and our revenue could be reduced.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a United States patent covering one of our product candidates even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for our licensed patents, we do not have the right to control prosecution, including filing with the USPTO, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming, and unsuccessful.

Competitors and other third parties may infringe, misappropriate, or otherwise violate our rights to owned and licensed patents, trade secrets, or other intellectual property. As a result, to discourage, prevent, or counter infringement, misappropriation, or unauthorized use, we may be required to file infringement or misappropriation claims or other intellectual property related proceedings, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke such parties to assert counterclaims against us alleging that we infringed their patents or that our asserted patents are invalid. In addition, in a patent infringement or other intellectual property related proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information or trade secrets could be compromised by disclosure during this type of litigation.

We may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in other contested proceedings such as opposition, derivation, reexamination, inter partes review, post-grant review, or interference proceedings in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding, or litigation could invalidate or reduce the scope of our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our intellectual property is threatened, it could dissuade companies from collaborating with us to license, develop, or commercialize current or future product candidates.

In the United States, the FDA does not prohibit clinicians from prescribing an approved product for uses that are not described in the product's labeling. Although use of a product directed by off-label prescriptions may infringe our method-of-treatment patents, the practice is common across medical specialties, particularly in the United States, and such infringement is difficult to detect, prevent, or prosecute.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability to develop, manufacture, market, and sell our product candidates and use our proprietary technologies without infringing or otherwise violating the intellectual property and other proprietary rights of third parties. There is a considerable amount of intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, claims by a third party for use, misappropriation, or infringement of their intellectual property rights regarding our products and technology. Claims of use, misappropriation, or infringement of intellectual property rights may arise from competitors or even from entities that may have patents but do not use or practice their patent. Our own patent portfolio may have no deterrent effect on entities that do not use of practice their own patent. Moreover, we may become party to future adversarial proceedings or litigation regarding our patent portfolio or the patents of third parties. Such proceedings could also include contested post-grant proceedings such as oppositions, inter partes review, reexamination, interference, or derivation proceedings before the USPTO or foreign patent offices.

The legal threshold for initiating litigation or contested proceedings is low, so even lawsuits or proceedings with a low probability of success might be initiated by a third party, which will require significant resources from us to defend. Litigation and contested proceedings can be expensive and time-consuming, and our adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we do. The risks of being involved in such litigation and proceedings may increase as our product candidates near commercialization and as we gain the greater visibility associated with being a public company. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. We may not be aware of all such intellectual property rights potentially relating to our product candidates and their uses.

Thus, we do not know with certainty that any of our product candidates, or our development and commercialization thereof, do not and will not infringe or otherwise violate any third party's intellectual property.

If we are found to infringe or otherwise violate a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing its products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we are able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us and could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease commercializing or practicing the infringing technology or product. 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, and could be forced to indemnify our customers or collaborators. A finding of infringement could also result in an injunction that prevents us from commercializing our product candidates or forces us to cease some of our business operations, which could materially 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 confidential information or trade secrets of third parties could also have negative impact on our business.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance, renewal, and annuity fees on any issued patent must be paid to the USPTO and foreign patent agencies in several stages or annually over the lifetime of our owned and licensed patents and patent applications. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment, and other similar provisions during the patent application process. In certain circumstances, we rely on our licensing partners to pay these fees to, or comply with, the procedural and documentary rules of the relevant patent agency. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it would have a material adverse effect on our business.

Certain aspects of our product candidates are protected by patents exclusively licensed from other companies or institutions. If these Licensors terminate their agreements with us or fail to maintain or enforce the underlying patents or licenses thereto, or we otherwise lose our rights to these patents, our competitive position and our market share in the markets for any of our licensed and approved products will be harmed.

A substantial portion of our patent portfolio is in-licensed. As such, we are party to license agreements and certain aspects of our business depend on patents and/or patent applications owned by other companies or institutions. For example, we hold exclusive licenses for patent families relating to OCU400, OCU410, OCU410ST, and OCU200, and an exclusive license in the United States, Europe, Japan, South Korea, Australia, China, and Hong Kong with respect to a mucosal COVID-19 vaccine.

Pursuant to the CU Agreement, which primarily relates to OCU200, we are responsible for and control the patent prosecution of all patent families licensed under the CU Agreement.

Pursuant to the SERI Agreement, which relates to NHR genes *NR1D1*, NR2E3 (OCU400), *RORA* (OCU410 and OCU410ST), *NUPR1*, and *NR2C1*, from and after December 19, 2017, we have the right to assume responsibility and control patent prosecution of licensed patent families relating to these NHR genes. Additionally, we are responsible for and control patent prosecution for any patent applications developed in connection with the SERI Agreement filed after December 19, 2017 that are owned jointly by us and SERI, or solely by us.

Pursuant to the WU Agreement, which relates to mucosal COVID-19 vaccines, Washington University maintains control of patent preparation, filing, prosecution, and maintenance, subject to our right to negotiate with WU after the first anniversary of the effective date of the WU Agreement to assume responsibility for and control of the prosecution and maintenance of the patent rights throughout the Mucosal Vaccine Territory in Washington University's name.

Our rights with respect to in-licensed patents and patent applications may be lost if the applicable license agreement expires or terminates. We are likely to enter into additional license agreements to in-license patents and patent applications as part of the development of our business in the future, under which we may not retain control of the preparation, filing, prosecution, maintenance, enforcement, and defense of such patents. If we are unable to obtain and maintain licenses to these patents and patent applications for any reason, our ability to develop and commercialize our product candidates may be materially harmed.

Our licensors may not successfully prosecute certain patent applications, the prosecution of which they control, under which we are licensed and on which our business depends. Even if patents are issued from these applications, our licensors may fail to maintain these patents, may decide not to pursue litigation against third-party infringers, may fail to prove infringement, or may fail to defend against counterclaims of patent invalidity or unenforceability. In some cases, our licensors may in-license certain patents licensed to us. If our licensors were to fail to maintain such licenses, we may need to obtain additional licenses with respect to the applicable product candidates.

Risks with respect to parties from whom we have obtained intellectual property rights may also arise out of circumstances beyond our control. In spite of our best efforts, our licensors might conclude that we have materially breached the license agreements and might therefore terminate the license agreements, thereby removing our ability to market products covered by these license agreements. If our license agreements are terminated, or if the underlying patents fail to provide the intended market exclusivity, such termination or failure may severely impact our business. Moreover, if our license agreements are terminated, our former licensors and/or assignors may be able to prevent us from utilizing the technology covered by the licensed patents and patent applications. This could have a materially adverse effect on our competitive business position and our business prospects.

Some intellectual property which we own or have licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for United States industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements and limit our ability to contract with non-United States manufacturers.

Some of the licenses or intellectual property rights that we own have been generated through the use of the United States government funding and may therefore be subject to certain federal regulations under the Bayh-Dole Act. To the best of our knowledge, our intellectual property for OCU400 for the treatment of *RHO* and other gene-agnostic mutations-associated with RP, is subject to the Bayh-Dole Act. As a result, the United States government may have certain rights to intellectual property embodied in these patents and patent applications. In general, the Bayh-Dole Act provides the United States government certain rights in inventions developed using a government funded program, such as the United States government's right to a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, under the Bayh-Dole Act, the United States government has the right to require any invention developed using the United States government funding to be granted exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations ("march-in rights"). Under the Bayh-Dole Act, the United States government also has the right to take title to inventions developed using a United States government funded program, if one fails to disclose the invention to the

government and fails to file an application to register the intellectual property within specified time limits. In addition, the United States government may acquire title to these inventions in any country in which a patent application is not filed within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements. In addition, the Bayh-Dole Act requires that any products subject to the Bayh-Dole Act be manufactured substantially in the United States. However, under the Bayh-Dole Act, this manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable efforts to manufacture the product substantially in the United States were unsuccessful, or that under the circumstances, domestic manufacture is not commercially feasible. Any exercise by the government of any of the foregoing rights under the Bayh-Dole Act may affect our competitive position, business, financial condition, results of operations, and prospects.

If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

Our agreements under which we license certain of our patent rights and a significant portion of the technology for our product candidates impose royalty and other financial obligations and other performance obligations. We may also enter into additional licensing and funding arrangements with third parties that may impose diligence, development, and commercialization timelines and milestone payment, royalty, insurance, and other obligations. If we fail to comply with our obligations under current or future license and collaboration agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture, or market any product that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could diminish the value of our products and product candidates. Termination of these agreements or reduction or elimination of our rights under these agreements may result in us 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.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and as with any agreements certain provisions in such agreements may be susceptible to different interpretations by the parties. 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 have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

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

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection or licenses, but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these

countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

We may be subject to claims by third parties asserting misappropriation of their intellectual property or claiming ownership of what we regard as our own intellectual property.

Many of our and our licensors' employees and contractors were previously employed at other biotechnology, medical device, or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and contractors do not use the proprietary information or know-how of their prior employer(s) 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 employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the 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 develops intellectual property that we regard as our own. Furthermore, we are unable to control whether our licensors have obtained similar assignment agreements from their own employees and contractors. Our and their assignment agreements may not be self-executing or may be breached, and we or our licensors may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensors fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel which could have a material adverse effect on our competitive business position and prospects. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products, which may not be available on commercially reasonable terms or may be barred from commercializing out technology or products. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation or other legal proceedings relating to intellectual property could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and may also have an advantage in such proceedings due to their more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our technology and product candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Detecting the disclosure or misappropriation of a trade secret and enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, many jurisdictions, both foreign and domestic, are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Risks Related to Our Common Stock

We do not currently intend to pay dividends on our common stock, and, consequently, your ability to achieve a return on your investment will depend on appreciation, if any, in the price of our common stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation, and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, the terms of the Loan and Security Agreement precludes us from paying cash dividends and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited to the appreciation of their stock. Accordingly, you may have to sell some or all of your shares of our common stock in order to generate cash flow from your investment. There is no guarantee that the common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

Sales of a substantial number of common stock by our stockholders in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of common stock in the public market, the market price of our common stock could decline. We had 291.4 million shares of common stock outstanding as of December 31, 2024, which were all freely tradable, without restriction, in the public market.

If a substantial number of shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline and we are unable to predict the effect that sales may have on the prevailing market price of our common stock.

Provisions in our charter documents and under Delaware law could discourage a takeover that stockholders may consider favorable and may lead to entrenchment of management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could significantly reduce the value of our shares to a potential acquiror or delay or prevent changes in control or changes in our management without the consent of our Board. The provisions in our charter documents include the following:

- a classified Board of Directors with three-year staggered terms, which may delay the ability of stockholders to change the membership of a majority of our Board;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- the exclusive right of our Board, unless the Board grants such right to the stockholders, to elect a director to fill a vacancy created by the expansion of the Board or the resignation, death, or removal of a director, which prevents stockholders from being able to fill vacancies on our Board;
- the prohibition on removal of directors without cause due to the classified Board;
- the ability of our Board to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquiror;
- the ability of our Board of Directors to alter our amended and restated bylaws without obtaining stockholder approval;
- the required approval of at least 66-2/3% of the shares entitled to vote to adopt, amend, or repeal our amended and restated bylaws or repeal certain provisions of our amended and restated certificate of incorporation;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- an exclusive forum provision providing that the Court of Chancery of the State of Delaware will be the exclusive forum for certain actions and proceedings;
- the requirement that a special meeting of stockholders may be called only by the chairman of the Board of Directors, the Chief Executive Officer, or the Board, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors; and
- advance notice procedures that stockholders must comply with in order to nominate candidates to our Board or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquiror from

conducting a solicitation of proxies to elect the acquiror's own slate of directors or otherwise attempting to obtain control of us.

We are also subject to the anti-takeover provisions contained in Section 203 of the Delaware General Corporation Law ("DGCL"). Under Section 203, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

Our sixth amended and restated certificate of incorporation, as amended, provides that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our sixth amended and restated certificate of incorporation, as amended, provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty, any action asserting a claim against us arising pursuant to the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. This exclusive forum provision would not apply to suits brought to enforce any liability or duty created by the Securities Act of 1933, as amended, or any other claim for which the federal courts have exclusive jurisdiction. These choice of forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage such lawsuits against us and our directors, officers, and other employees. By agreeing to this provision, however, stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. If a court were to find the choice of forum provisions in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

Our failure to meet the continued listing requirements of Nasdaq could result in a delisting of our common stock.

We must continue to satisfy Nasdaq continued listing requirements, including, among other things, certain corporate governance requirements and a minimum closing bid price requirement of $1.00 per share. If a company fails for 30 consecutive business days to meet the $1.00 minimum closing bid price requirement, Nasdaq will send a deficiency notice to the company, advising that it has been afforded a "compliance period" of 180 calendar days to regain compliance with the applicable requirements.

On December 31, 2024, we received a deficiency letter from Nasdaq notifying us that, for the last 30 consecutive business days, the closing bid price for our common stock was below the minimum $1.00 per share required for continued listing on Nasdaq pursuant to the minimum closing bid price requirement. The Nasdaq deficiency letter had no immediate effect on the listing of our common stock. In accordance with Nasdaq Listing Rule 5810(c)(3)(A), we have been given 180 calendar days, or until June 30, 2025, to regain compliance with the minimum closing bid price requirement by causing our stock to close above $1.00 for a minimum of 10 consecutive trading days. If we do not regain compliance with the minimum closing bid price requirement by June 30, 2025, we may be afforded a second 180 calendar day period to regain compliance. To qualify, we would be required to meet the continued listing requirement for market value of publicly held shares and all other initial listing standards for Nasdaq, except for the minimum bid price requirement. In addition, we would be required to notify Nasdaq of our intent to cure the deficiency during the second compliance period.

We can provide no assurance that we will be able to remain in compliance with other Nasdaq continued listing requirements. A delisting of our common stock from Nasdaq could materially reduce the liquidity of our common stock, impairing your ability to sell or purchase shares of our common stock when you wish to do so, and could result in a corresponding material reduction in the price of our common stock. In addition, delisting could harm our ability to raise capital through alternative financing sources on terms acceptable to us, or at all, and may result in the potential loss of confidence by investors and employees. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow the common stock to become listed again, stabilize the market price or improve the liquidity of the common stock, prevent the common stock from dropping below the Nasdaq minimum bid price requirement, or prevent future non-compliance with Nasdaq's listing requirements.

The trading price of the shares of our common stock could be highly volatile, and purchasers of our common stock could incur substantial losses.

Our stock price has been, and will likely continue to be volatile. The stock market in general and the market for stock of biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above their purchase price. The market price for our common stock may be influenced by those factors discussed in this "Risk Factors" section and many others, including:

- our ability to enroll subjects in our ongoing and planned clinical trials;
- the results of our clinical trials and preclinical studies, and the results of trials of our competitors or those of other companies in our market sector;
- regulatory approval of our product candidates, or limitations to specific label indications or patient populations for use, or changes or delays in the regulatory review process;
- the level of expenses related to any of our product candidates or clinical development programs;
- regulatory developments in the United States and foreign countries;
- reports of adverse events in any of our products, competing biologics, or gene therapy products;
- changes in the structure of healthcare payment systems, especially in light of current reforms to the United States healthcare system;
- the success or failure of our efforts to acquire, license, or develop additional product candidates;
- innovations or new products developed by us or our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, or capital commitments;
- manufacturing, supply, or distribution delays or shortages;
- any changes to our relationship with any manufacturers, suppliers, licensors, future collaborators, or other strategic partners;
- achievement of expected product sales and profitability;
- variations in our financial results or those of companies that are perceived to be similar to ours;
- market conditions in the biopharmaceutical sector and issuance of securities analysts' reports or recommendations;
- trading volume of our common stock;
- an inability to obtain additional funding;
- sales of our stock by insiders and stockholders or the perception that such sales could occur;
- our ability to effectively manage our growth;
- ineffectiveness of our internal control over financial reporting;
- additions or departures of key personnel, including major changes in our board or management;
- intellectual property, product liability, or other litigation against us; and
- general economic, industry, market conditions, and other events or factors, many of which are beyond our control.

In addition, in the past, stockholders have initiated class action lawsuits against biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, including the litigation instituted against us in our current class action lawsuits, could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition, and results of operations. See the section of this Annual Report for the fiscal year ended December 31, 2024 entitled, "Legal Proceedings."

If securities or industry analysts do not publish research or reports or publish unfavorable research or reports about our business, our stock price and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us, our business, our market, or our competitors. We currently have research coverage by six securities and industry

analysts. If one or more of the analysts who currently or in the future may cover us downgrades our stock, our stock price would likely decline. If one or more of these analysts ceases to cover us or fails to regularly publish reports on us, interest in our stock could decrease, which could cause our stock price or trading volume to decline.

General Risk Factors

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

We are highly dependent on the research and development, clinical, and business development expertise of Shankar Musunuri, Ph.D., MBA, our Chief Executive Officer, Chairman of the Board, and Co-Founder, as well as the other principal members of our management, scientific, and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees.

Recruiting and retaining qualified scientific, clinical, manufacturing, legal, financial, and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development, and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of, and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain, or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We expect to expand our development, regulatory, and manufacturing capabilities and potentially implement sales, marketing, and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs, manufacturing, sales, marketing, and distribution. For example, we have completed renovating an existing facility into a current GMP facility in accordance with the FDA's regulations in support of NeoCart manufacturing for Phase 3 clinical trial material. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational, and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of our attention to managing these growth activities. Due to our limited financial resources and our limited experience in managing such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Our inability to manage the expansion of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced, and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

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

As a public company we have incurred, and will continue to incur, significant legal, accounting, and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002 ("Sarbanes-Oxley"), the Dodd-Frank Wall Street Reform, the Consumer Protection Act, the listing requirements of Nasdaq, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We have had to hire additional accounting, finance, and other personnel in connection with our efforts to comply with the requirements of being a public company and our management and other personnel devote a substantial amount of time towards maintaining compliance with these requirements. These requirements increase our legal and financial compliance costs and make some activities more time-consuming and costly. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their

application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

In addition, Sarbanes-Oxley, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of Sarbanes-Oxley, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, the SEC has adopted additional rules and regulations in these areas, such as mandatory "say on pay" voting requirements that are applicable to us. Stockholder activism, the current political environment, and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition, and results of operations. The increased costs could impact our results of operations and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our Board, our board committees, or as executive officers.

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

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological agents coverage and our commercial general liability policy specifically excludes coverage for damages and fines arising from biological agents. Accordingly, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws, regulations, and permitting requirements. These current or future laws, regulations, and permitting requirements may impair our research, development, or production efforts. Failure to comply with these laws, regulations, and permitting requirements also may result in substantial fines, penalties, or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health, and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, investors may lose confidence in our financial reporting, and the trading price of our common stock may decline.

Pursuant to Section 404 of Sarbanes-Oxley, our management is required to report upon the effectiveness of our internal control over financial reporting. This assessment includes disclosure of any material weaknesses identified by our management in internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation. If we are unable to conclude that our internal control over financial reporting is effective, investors may lose confidence in our financial reporting and the trading price of our common stock may decline.

As part of our annual review of the effectiveness of our internal control over financial reporting as of December 31, 2024, management has concluded that our internal control over financial reporting was effective. A material weakness, which was previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2023, and in our Quarterly Reports on Form 10-Q for the periods ended March 31, June 30 and September 30, 2024, has been remediated after newly implemented controls had been operating effectively for a sufficient period of time.

Although this material weakness was remediated as of December 31, 2024, we cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations, or cash flows.

We continue to implement measures designed to improve our internal controls over financial reporting. Failure to implement and maintain effective internal control over financial reporting or a material weakness in our internal control over financial reporting could result in an increased probability of fraud, litigation from our shareholders, reduction in our ability to obtain financing, and require additional expenditures to remediate. Additionally, if we are unable to conclude that our internal control over financial reporting is effective, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC, or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability for our product candidates and may have to limit our commercialization.

The use of our product candidates in clinical trials, and the sale of any of our product candidates for which we obtain regulatory approval, exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical or biotechnology companies, or others selling or otherwise coming into contact with our products. For example, we may be sued if any product candidate we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing, or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourself against these claims, we will incur substantial liabilities or be required to limit development or commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of merit or eventual outcome, liability claims may result in:

- loss of revenue from decreased demand for our products and/or product candidates;
- impairment of our business reputation or financial stability;
- costs of related litigation;
- substantial monetary awards to patients or other claimants;
- exhaustion of any available insurance and our capital resources;
- diversion of management attention;
- withdrawal of clinical trial participants and potential termination of clinical trial sites or entire clinical programs;
- the inability to commercialize our product candidates;
- significant negative media attention;

- decrease in our stock price;
- initiation of investigations and enforcement actions by regulators; or
- product recalls, withdrawals, revocation of approvals, or labeling, marketing, or promotional restrictions.

While we currently hold product liability insurance coverage in an amount that we believe is customary for similarly situated companies, the amount of that coverage may not be adequate. We may need to increase our insurance coverage as we continue to conduct our clinical trials. We will need to further increase our insurance coverage if we commence commercialization of any of our product candidates for which we obtain marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on therapeutics that had unanticipated side effects. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. A successful product liability claim or series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business and our prospects.

Our internal computer systems or those of our development collaborators, third-party CDMOs, or other contractors or consultants may fail or suffer cybersecurity incidents, data breaches, or other disruptions, which could result in a material disruption of our product development programs and cause our business and operations to suffer.

Our internal computer systems and those of our CDMOs and other contractors and consultants are vulnerable to cybersecurity incidents or data breaches and damage from computer viruses, unauthorized access, ransomware, social engineering attacks (including phishing attacks), natural disasters, terrorism, war, and telecommunication and electrical failures. While we have not experienced any material system failure, accident, or cybersecurity incident or data breach to date, we, like other companies in our industry, have experienced and may in the future continue to experience, threats and cybersecurity incidents relating to our and our third-party vendors' information systems. If a material event were to occur and cause interruptions in our operations, it could result in a material disruption of our business operations and product candidate development and, if any of our product candidates are approved, commercialization programs. Likewise, we intend to rely on third parties to manufacture our product candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business and operations. To the extent that any disruption or cybersecurity incident or other data breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, the further development and commercialization of our product candidates could be delayed, and our reputation could be harmed. In addition, there are known cyberattacks against biotechnology companies engaged in the development of therapeutic or vaccine products addressing COVID-19.

Additionally, our business processes personal data, including some data related to health. When conducting clinical trials, we face risks associated with collecting trial participants' data, especially health data, in a manner consistent with applicable laws and regulations. We also face risks inherent in handling large volumes of data and in protecting the security of such data. We could be subject to attacks on our systems by outside parties or fraudulent or inappropriate behavior by our service providers or employees. Third parties may also gain access to our systems using stolen or inferred credentials, computer malware, viruses, spamming, phishing attacks, or other means, and may use such access to obtain personal data. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures.

Any failure by us or our service providers, collaborators, consultants, contractors or partners to detect, prevent, respond to or mitigate data breaches or cybersecurity incidents, compromises, or improper access to, use of, or inappropriate disclosure of any of this information or other confidential or sensitive information, including patients' personal data, or the perception that any such failure has occurred, could result in claims, litigation, regulatory investigations and other proceedings, significant liability under state, federal and international law, and other financial, legal or reputational harm to us, including class action lawsuits from affected individuals. Further, such failures or perceived failures could result in liability and a material disruption of our development programs and our business operations, which could lead to significant delays or setbacks in our research, delays to commercialization of our product candidates, lost revenues or other adverse consequences, any of which could have a material adverse effect on our business, results of operations, financial condition, prospects and cashflow. For example, the loss or alteration of clinical trial data from future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

Cybersecurity incidents or data breaches could subject us to individual or consumer class action litigation and governmental investigations and proceedings by federal, state, and local regulatory entities in the United States and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. Additionally, applicable laws and regulations relating to privacy, data protection or cybersecurity, external contractual commitments and internal privacy and security policies may require us to notify relevant stakeholders if there has been a cybersecurity incident or data breach, including affected

individuals, business partners and regulators. Such disclosures are costly, and the disclosures or any actual or alleged failure to comply with such requirements could lead to a materially adverse impact on the business, including negative publicity, a loss of confidence in our services or security measures by our business partners or breach of contract claims. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages if we fail to comply with applicable data protection laws, privacy policies or other data protection obligations related to information cybersecurity incidents, compromises, or security breaches. Further, although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any cybersecurity incident or data breach.

We face risks related to our collection and use of data, which could result in investigations, inquiries, litigation, fines, legislative and regulatory action, and negative press about our privacy and data protection practices.

As our operations and business grow, we may become subject to or be affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities, including various domestic and international privacy and security regulations. The legislative and regulatory landscape for privacy and data protection continues to evolve. In the United States, at the federal level, failing to take appropriate steps to keep consumers' personal information secure may constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the FTC Act, 15 U.S.C § 45(a). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business and the cost of available tools to improve security and reduce vulnerabilities. Through executive and legislative action, the federal government has also taken steps to restrict data transactions involving certain sensitive data categories – including health data, genetic data, and biospecimens – with persons affiliated with China, Russia, and other countries of concern.

In addition, certain U.S. states have adopted privacy and security laws and regulations that may be more stringent than applicable federal law. For example, the CCPA, which took effect on January 1, 2020, established a comprehensive privacy framework for covered businesses by creating an expanded definition of personal information, establishing new data privacy rights for consumers in the State of California, 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 California Privacy Rights Act of 2020 ("CPRA"), effective January 1, 2023, expanded the CCPA's requirements, including expanding consumers' rights with respect to certain sensitive personal information, broadening the application of the CCPA to personal information of business representatives and employees and establishing a new regulatory agency to implement and enforce the law. While clinical trial data and information governed by HIPAA are currently exempt from the current version of the CCPA, other personal information may be applicable and possible changes to the CCPA may broaden its scope.

Similar laws have been passed in numerous other states. Other states have proposed new privacy laws which, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. The existence of comprehensive privacy laws in different states in the country would make our compliance obligations more complex and costly and may increase the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. There are also states that are specifically regulating health information. For example, Washington's My Health My Data Act ("WMHMDA"), which went into effect March 31, 2024, regulates the collection and sharing of health information. WMHMDA also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain specific types of information. For example, a small number of states have passed laws that regulate biometric data specifically. These various privacy and security laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. State laws are changing rapidly and there have been discussions in the United States Congress of a new comprehensive federal data privacy law to which we may likely become subject, if enacted.

We may also in the future be subject to data protection laws and regulations of other jurisdictions, such as the GDPR. The GDPR in the EU and the UK, which have been incorporated into their respective laws, impose stringent requirements on the processing of health and other sensitive data. These requirements encompass: (i) providing information to individuals regarding data processing activities; (ii) ensuring a legal basis or condition applies to the processing of personal data and, where applicable, obtaining consent from individuals to whom the data processing relates; (iii) responding to data subject requests; (iv) imposing requirements to notify the competent national data protection authorities and data subjects of personal data breaches; (v) implementing safeguards in connection with the security and confidentiality of the personal data; (vi) accountability requirements; and (vii) taking certain measures when engaging third-party processors. In the event that we are subject to or affected by privacy and data protection laws, including the CCPA, CPRA, or GDPR and other domestic or

international privacy and data protection laws, we may expend significant resources to comply with such laws, and any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

All of these evolving compliance and operational requirements impose significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. In addition, such requirements may require us to modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects. Any failure or perceived failure by us to comply with any applicable federal, state or foreign laws and regulations relating to data privacy and security could result in damage to our reputation, as well as proceedings or litigation by governmental agencies or other third parties, including class action privacy litigation in certain jurisdictions, which would subject us to significant fines, sanctions, awards, injunctions, penalties or judgments. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

The use of new and evolving technologies, such as artificial intelligence, in our business may result in spending material resources and presents risks and challenges that can impact our business including by posing security and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability

We may use and integrate artificial intelligence into our business processes, and this innovation presents risks and challenges that could affect its adoption, and therefore our business. If we enable or offer solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability. The use of certain artificial intelligence technology can give rise to intellectual property risks, including compromises to proprietary intellectual property and intellectual property infringement. Additionally, we expect to see increasing government and supranational regulation related to artificial intelligence use and ethics, which may also significantly increase the burden and cost of research, development and compliance in this area. For example, the EU's Artificial Intelligence Act ("AI Act") — the world's first comprehensive AI law — which entered into force on August 1, 2024 and most provisions of which will become effective on August 2, 2026. This legislation imposes significant obligations on providers and deployers of high-risk artificial intelligence systems, and encourages providers and deployers of artificial intelligence systems to account for EU ethical principles in their development and use of these systems. If we develop or use AI systems that are governed by the AI Act, it may necessitate ensuring higher standards of data quality, transparency, and human oversight, as well as adhering to specific and potentially burdensome and costly ethical, accountability, and administrative requirements. Furthermore, in the U.S., a number of states have proposed and passed laws regulating various uses of AI, and federal regulators have issued guidance affecting the use of AI in regulated sectors.

The rapid evolution of artificial intelligence will require the application of significant resources to design, develop, test and maintain our products and services to help ensure that artificial intelligence is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. Our vendors may in turn incorporate artificial intelligence tools into their offerings, and the providers of these artificial intelligence tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of artificial intelligence, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our research, product candidates, and the diseases those product candidates and investigational medicines are being developed to treat. Social media practices in the biotechnology industry and the FDA's regulation of social media continues to evolve. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us. For example, our employees or agents may use social media channels to inadvertently provide inaccurate or misleading information about our product candidates. If regulators become aware of such disclosures, they may take administrative or enforcement action against us. There is also a risk that third parties will use social media to disseminate inaccurate or misleading information about us or our product candidates. If this occurs, we may not be able to adequately defend our business or the public's perception of us or our product candidates, particularly given restrictions on what we may say about our product candidates prior to FDA approval. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

Evolving expectations around corporate responsibility practices, specifically related to environmental, social and governance ("ESG") matters, may expose us to reputational and other risks.

Investors, stockholders, customers, suppliers and other third parties are increasingly focusing on ESG and corporate social responsibility endeavors and reporting. Certain institutional investors, investment funds, other influential investors, customers, suppliers and other third parties are also increasingly focused on ESG practices. Companies that do not adapt to or comply with the evolving investor or stakeholder expectations and standards, or which are perceived to have not responded appropriately, may suffer from reputational damage and result in the business, financial condition, and/or stock price of a company being materially and adversely affected. Further, this increased focus on ESG issues may result in new regulations and/or third party requirements that could adversely impact our business, or certain shareholders reducing or eliminating their holdings of our stock. Additionally, an allegation or perception that we have not taken sufficient action in these areas could negatively harm our reputation.

We are and expect to continue to be a "smaller reporting company" as defined in the Exchange Act, and have elected and expect to continue to elect to take advantage of certain of the scaled disclosures available to smaller reporting companies, including reduced disclosure obligations regarding executive compensation.

We are and expect to continue to be a "smaller reporting company" as defined in the Exchange Act and have elected and expect to continue to elect to take advantage of certain of the scaled disclosures available to smaller reporting companies, including reduced disclosure obligations regarding executive compensation. These exemptions and reduced disclosures in our SEC filings due to our status as a smaller reporting company also mean our auditors are not required to audit our internal control over financial reporting for so long as we report less than $100 million in annual revenues for the most recent fiscal year and may make it harder for investors to analyze our results of operations and financial prospects. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our common stock price may be more volatile. We will remain a smaller reporting company until our public float exceeds $250 million or our annual revenues exceed $100 million with a public float greater than $700 million.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

Cybersecurity Risk Management and Strategy

We understand the importance of assessing, identifying, and managing risks associated with cybersecurity threats. Cybersecurity processes designed to assess, identify and manage risks from cybersecurity threats have been incorporated into our operations as a part of our overall risk assessment process.

To help us defend against, detect and respond to risks from cybersecurity threats, we engage a third-party cybersecurity firm to assist with aspects of our cybersecurity program including, but not limited to, network monitoring, cloud system monitoring, and employee cybersecurity awareness training. Training topics include how to escalate suspicious activities including phishing, viruses, spams, insider threats, suspect human behaviors or safety issues. We also maintain cyber liability insurance.

Our processes also include assessing cybersecurity risks associated with our use of third-party services providers in the normal course of business use. We assess cybersecurity considerations in the selection and oversight of our third-party services providers, including due diligence on the third parties that have access to our systems.

There have not been any risks from cybersecurity threats, including as a result of any cybersecurity incidents, which have materially affected or are reasonably likely to materially affect our Company, including our business strategy, results of operations, or financial condition. Refer to "Item 1A. Risk factors" in this Annual Report, including "Our internal computer systems or those of our development collaborators, third-party CDMOs, or other contractors or consultants may fail or suffer cybersecurity incidents, data breaches, or other disruptions, which could result in a material disruption of our product development programs and cause our business and operations to suffer," for additional discussion about cybersecurity-related risks.

Cybersecurity Governance

Cybersecurity is an important part of our risk management processes. Our Associate Vice President of IT & Facilities is responsible for overseeing the cybersecurity risk management program. He has over 20 years of IT management, cybersecurity, and information governance experience. In order to monitor and appropriately escalate cybersecurity risks, our Associate Vice President of IT & Facilities receives reports on a monthly basis, and more frequently as appropriate, from our third-party cybersecurity vendor.

Our Board's role in risk oversight is consistent with our leadership structure, with management having day-to-day responsibility for assessing and managing our risk exposure and our Board actively overseeing the management of our risks both at the Board and Committee level. The Board conducts its risk oversight by receiving reports from each of the Committees and our executive officers regarding our risk identification, risk management, and risk mitigation strategies with respect to areas of potential material risk, including cybersecurity risk. The Board has delegated to the Audit Committee of the Board primary responsibility for overseeing risks from cybersecurity threats. Our Associate Vice President of IT & Facilities briefs the Audit Committee on our cybersecurity risk management program on a quarterly basis, using risk assessment reports from our third-party cybersecurity vendor. The briefing includes discussion of management's actions to identify and detect threats, as well as planned actions in the event of a response or recovery situation.

Item 2. Properties

Our properties are located in Malvern, Pennsylvania and other locations, including our corporate headquarters, which consist of approximately 28,488 square feet of leased office space, and our current GMP facility, which consists of approximately 16,401 square feet of laboratory and future manufacturing space. Our corporate headquarters has an initial term of approximately seven years and includes options to extend the leases for up to 10 years, which we have not elected to account for since it is not reasonably certain that we will exercise such option. Our current GMP facility has an initial term of seven years and includes an option to extend the lease for up to five years, which we have elected to account for since it is reasonably certain that we will exercise such option. We lease two other general use facilities within the United States, which have initial terms of two to three years and contain no option to extend. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional space will be available as and when needed.

Item 3. Legal Proceedings.

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this Annual Report, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

For a discussion of legal proceedings, see Note 14 in the notes to the consolidated financial statements included elsewhere in this Annual Report. This discussion is incorporated herein by reference.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock is traded on the Nasdaq under the symbol "OCGN."

Holders

As of February 26, 2025, we had 292.0 million shares of common stock outstanding held by approximately 26 stockholders of record. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in "street" name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

We have not declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings, if any, to fund our operations and do not anticipate declaring or paying any cash dividends for the foreseeable future. Furthermore, our ability to pay cash dividends is currently restricted by the terms of our Loan and Security Agreement with Avenue 1 and Avenue 2. As a result, we anticipate that only the appreciation of the price of our common stock, if any, will provide a return to investors for at least the foreseeable future.

Unregistered Sales of Equity Securities and Use of Proceeds

None.

Issuer Purchases of Equity Securities

During the quarter ended December 31, 2024, we did not repurchase any shares of our common stock.

Securities Authorized for Issuance Under Equity Compensation Plans

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the consolidated financial statements and the notes thereto included elsewhere in this Annual Report. Some of the information contained in this discussion and analysis, including information with respect to our plans and strategy for our business and related financing, include forward-looking statements that involve risks, uncertainties, and assumptions. These statements are based on our beliefs and expectations about future outcomes and are subject to risks and uncertainties that could cause our actual results to differ materially from anticipated results. Except as required by law, we undertake no obligation to publicly update these forward-looking statements, whether as a result of new information, future events, or otherwise. You should read the "Risk Factors" and "Special Note Regarding Forward-Looking Statements" sections of this Annual Report for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics and vaccines that improve health and offer hope for patients across the globe.

Our technology pipeline includes:

- **Modifier Gene Therapy Platform** — Based on the use of NHRs, we believe our modifier gene therapy platform has the potential to address many retinal diseases, including rare genetic diseases such as RP (OCU400), with a gene-agnostic approach. We also believe our modifier gene therapy platform has the potential to address multifactorial retinal diseases including dAMD, which affects millions of patients in the United States alone, using OCU410, and Stargardt disease, which is a rare genetic disease, using OCU410ST. We are actively recruiting patients in the United States and Canada in the Phase 3 liMeliGhT clinical trial for OCU400 for the treatment of RP and are on track to complete enrollment in the first half of 2025. In January 2025, we announced positive two year data for multiple mutations from the Phase 1/2 clinical trial of OCU400. In February 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU400 ATMP classification. OCU410 completed dosing in Phase 2 of the Phase 1/2 ArMaDa clinical trial for the treatment of GA, an advanced form of dAMD. Positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the OCU410 Phase 1/2 ArMaDa clinical trial included: no drug-related SAEs, reduced lesion growth, preservation of retinal tissue, and—most importantly—there was a positive effect on the functional visual measure of LLVA. In October 2024, the DSMB for the OCU410ST GARDian clinical trial approved enrollment for the second phase of the Phase 1/2 clinical trial. In February 2025, we announced that alignment has been reached with the FDA to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a BLA submission. Additionally, the EMA granted OMPD for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, RP19, and CORD3. Both OCU410 and OCU410ST studies have completed the low, medium, and high dose cohorts in the Phase 1 part of the Phase 1/2 trials to date, with OCU410 completing dosing in the Phase 2 part of the Phase 1/2 trials.
- **Novel Biologic Therapy for Retinal Diseases** — OCU200 is a novel fusion protein consisting of two human proteins, tumstatin and transferrin. OCU200 possesses unique features which potentially enable it to treat vascular complications of DME, DR, and wet AMD. Tumstatin is the active component of OCU200 and binds to integrin receptors, which play a crucial role in disease pathogenesis. Transferrin is expected to facilitate the targeted delivery of tumstatin into the retina and choroid and potentially help increase the interaction between tumstatin and integrin receptors. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and currently we are actively recruiting patients.
- **Regenerative Medicine Cell Therapy Platform** — Our Phase 3-ready regenerative cell therapy platform technology, which includes NeoCart (autologous chondrocyte-derived neocartilage), is being developed for the repair of knee cartilage injuries in adults. We received concurrence from the FDA on the confirmatory Phase 3 trial design and have completed renovating an existing facility into a current GMP facility to support clinical study and initial commercial launch. This facility is needed to generate patient-specific NeoCart implant from chondrocytes derived from knee biopsy. We intend to initiate the Phase 3 trial contingent on adequate availability of funding.
- **Inhaled Mucosal Vaccine Platform** — Our next-generation, inhaled mucosal vaccine platform includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine. We have completed IND-enabling studies and GMP manufacturing of clinical trial material for OCU500. In January 2025, we announced that the IND application is in effect and NIAID intends to

initiate a Phase 1 clinical trial in the second quarter of 2025. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

Modifier Gene Therapy Platform

We are developing a modifier gene therapy platform designed to fulfill unmet medical needs related to retinal diseases, including IRDs, such as RP, Stargardt disease; and multifactorial diseases such as dAMD. Our modifier gene therapy platform is based on the use of NHRs, which have the potential to achieve homeostasis — the basic biological processes in the retina to restore a healthy state from a diseased state. Unlike single gene replacement therapies, which only target one genetic mutation, our modifier gene therapy platform, through its use of NHRs, represents a unique, gene-agnostic approach designed to address not just the mutated gene but provide a molecular "reset" of health and survival of gene networks. OCU400, our lead product candidate in our modifier gene therapy platform, has received ODD from the FDA for RP and LCA, a RMAT designation for the treatment of RP associated with *NR2E3* and *RHO* mutations from the FDA, and OMPD from the EC, based on the recommendation of the EMA, for RP and LCA. These broad ODD, RMAT, and OMPD designations further support the broad (gene-agnostic) therapeutic potential of OCU400 to treat RP associated with mutations in multiple genes.

The OCU400 Phase 3 liMeliGhT clinical trial is currently underway, with enrollment on track to meet our target submission of a BLA and MAA in mid-2026.

In August 2024, we received notification from the FDA that we could begin our EAP for the treatment of adult patients with RP with OCU400. This program is available for patients with early, intermediate to advanced RP with at least minimal retinal preservation who may benefit from the mechanism of action of OCU400 prior to approval of the BLA.

We also received approval from Health Canada to initiate a Phase 3 trial for OCU400 for the treatment of RP. The Health Canada trial will run in parallel with the United States FDA trial, expediting the ability to potentially provide a gene-agnostic treatment option to approximately 110,000 patients in the United States and Canada.

In January 2025, we announced positive two-year long-term data across multiple mutations from the Phase 1/2 clinical trial of OCU400, which demonstrated a durable and statistically significant (p=0.005) improvement in visual function (LLVA) in all evaluable treated subjects at two years when compared to untreated eyes. 100% (10/10) of treated evaluable subjects demonstrated improvement or preservation in visual function compared to untreated eyes. Also, treated eyes with multiple mutations and *RHO* subjects demonstrated a statistically significant (p=0.005) improvement in visual function when compared to untreated eyes. Treated eyes in *RHO* subjects (who meet Intent-to-treat criteria) for OCU400 Phase 3 trial demonstrated a statistically significant improvement (p=0.01, N=4) with around two-line gain in visual function (LLVA) at two years when compared to untreated eyes.

In February 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU400 ATMP classification. ATMP classification is granted to medicines that can offer groundbreaking opportunities for the treatment of disease and accelerates the regulatory review timeline of this potential one-time gene therapy for life.

OCU410 and OCU410ST are being developed utilizing the nuclear receptor gene *RORA*, for the treatment of GA secondary to dAMD and Stargardt disease, respectively. OCU410 is a potential one-time, curative therapy with a single sub-retinal injection. OCU410 targets multiple pathways associated with AMD pathogenesis, in contrast to products currently approved or under development that treat only one cause of GA, require multiple injections per year, and have safety considerations. OCU410ST has received ODD from the FDA for the treatment of *ABCA4*-associated retinopathies, including Stargardt disease. OCU410ST has also received OMPD from the EMA for the treatment of *ABCA4*-associated retinopathies, including Stargardt disease, RP19, and CORD3. In March 2025, we announced that the EC has provided a positive opinion from the EMA Committee for Advanced Therapies for OCU410 and OCU410ST ATMP classification.

OCU410 has completed dosing in the Phase 2 of the Phase 1/2 ArMaDa clinical trial. OCU410ST has completed Phase 1 of the Phase 1/2 GARDian clinical trial, and the DSMB has approved enrollment for the second phase of the Phase 1/2 GARDian clinical trial.

In November 2024, we announced positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the Phase 1/2 ArMaDa clinical trial for GA. The key findings included: no drug-related SAEs, reduced lesion growth, preservation of retinal tissue, and a positive effect on the functional visual measure of LLVA. In February 2025, we announced dosing has been completed in Phase 2 of the OCU410 ArMaDa clinical trial. Phase 2 was a randomized expansion phase in which 51 subjects were randomized in a 1:1:1 ratio to either one of two OCU410 dose groups or to an untreated control group.

In October 2024, we announced that the DSMB had approved proceeding to Phase 2 using high and medium doses. In November 2024, we announced preliminary efficacy and safety data from evaluable subjects at six months from the Phase 1 dose-escalation portion of the OCU410ST Phase 1/2 GARDian clinical trial, which demonstrated an 84% reduction in atrophic lesion growth in treated eyes versus untreated fellow eyes.

In February 2025, we announced that alignment has been reached with the FDA to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a BLA submission. The Phase 2/3 clinical trial will randomize 51 subjects, 34 of whom will receive a single, subretinal, 200-µL injection of OCU410ST at a concentration of 1.5×10^{11} vector genomes (vg)/mL in the eye with worse visual acuity, and 17 of whom will serve as untreated controls. The primary endpoint in the clinical trial is change in atrophic lesion size. Secondary endpoints include visual acuity as measured by best corrected visual acuity (BCVA) and LLVA compared to untreated controls. One-year data will be utilized for the BLA filing.

Novel Biologic Therapy for Retinal Diseases

We are developing OCU200, which is a novel fusion protein containing parts of human transferrin and tumstatin. OCU200 is designed to treat DME, DR, and wet AMD. The first patient was dosed in the OCU200 Phase 1 clinical trial in January 2025, and we are actively recruiting patients for the Phase 1 clinical trial. The OCU200 Phase 1 clinical trial is a multicenter, open-label, dose-escalation study to assess drug safety via intravitreal injection in three cohorts: low dose (0.025 mg), medium dose (0.05 mg), and high dose (0.1 mg). All subjects will receive a total of two intravitreal injections of OCU200 six weeks apart. Patient follow-up will take place up to three months after the last injection.

Regenerative Medicine Cell Therapy Platform

NeoCart is a Phase 3-ready, regenerative cell therapy technology that combines breakthroughs in bioengineering and cell processing to enhance the autologous cartilage repair process. NeoCart is a three-dimensional tissue-engineered disc of new cartilage that is manufactured by growing the patient's own chondrocytes, the cells responsible for maintaining cartilage health. Current surgical and nonsurgical treatment options for knee cartilage injuries in adults are limited in their efficacy and durability. In prior clinical studies, Phase 2 and Phase 3, NeoCart has shown potential to accelerate healing, reduce pain, and provide regenerative native-like cartilage strength with durable benefits post transplantation. NeoCart was shown to be generally well-tolerated and demonstrated greater clinical efficacy than microfracture surgery at two years after treatment. Based on this clinical benefit, the FDA granted a RMAT designation to NeoCart for the repair of full-thickness lesions of knee cartilage injuries in adults. Additionally, we received concurrence from the FDA on the confirmatory Phase 3 trial design where chondroplasty will be used as a control group. We have completed renovating an existing facility into a GMP facility in accordance with the FDA's regulations in support of NeoCart manufacturing for personalized Phase 3 trial material. We intend to initiate the Phase 3 trial contingent on adequate availability of funding.

Inhaled Mucosal Vaccine Platform

We are party to the WU License Agreement with Washington University, pursuant to which we licensed the rights to develop, manufacture, and commercialize a mucosal COVID-19 vaccine for the prevention of COVID-19 in the Mucosal Vaccine Territory. In addition, we internally developed technology related to the flu and COVID-19's vaccine design and filed intellectual property. We are developing a next-generation, inhalation-based mucosal vaccine platform based on a novel ChAd vector, which includes OCU500, a COVID-19 vaccine; OCU510, a seasonal quadrivalent flu vaccine; and OCU520, a combination quadrivalent seasonal flu and COVID-19 vaccine. Our inhaled mucosal vaccine platform is driven by our conviction to serve a major public health concern, which requires the endorsement and support of government funding in order to develop and ultimately commercialize our vaccine candidates. As these vaccine candidates are being developed to be administered via inhalation, we believe they have the potential to generate rapid local immune response in the upper airways and lungs, where viruses enter and infect the body. We believe this novel delivery route may help reduce or prevent infection and transmission as well as provide protection against new virus variants. In October 2023, OCU500 was selected by the NIAID Project NextGen for inclusion in clinical trials. OCU500 will be tested via two different mucosal routes, inhalation and intranasal delivery. NIAID intends to initiate a Phase 1 clinical trial after IND clearance. We are continuing discussions with relevant government agencies as well as strategic partners regarding developmental funding for our OCU510 and OCU520 platforms.

Financial Operations Overview

We have not generated revenue from our product candidates to date and have incurred net losses in each year since inception. We expect to continue to incur net losses until our product candidates, if approved, are successfully commercialized. We

incurred net losses of approximately $54.1 million and $63.1 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $340.2 million and a cash balance of $58.5 million. Substantially all of our net losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

Segment Information

As of December 31, 2024, we viewed our operations and managed our business as one operating segment consistent with how our chief operating decision-maker, our Chief Executive Officer, makes decisions regarding resource allocation and assessing performance. As of December 31, 2024, substantially all of our assets were located in the United States. Our headquarters are located in Malvern, Pennsylvania.

Research and development expense

Research and development costs are expensed as incurred. These costs consist of internal and external expenses, as well as depreciation expense on assets used within our research and development activities. Internal expenses include the cost of salaries, benefits, and other related costs, including stock-based compensation, for personnel serving in our research and development functions, as well as allocated rent and utilities expenses. External expenses include development, clinical trials, patent costs, and regulatory compliance costs incurred with research organizations, contract manufacturers, and other third-party vendors. License fees paid to acquire access to proprietary technology are expensed to research and development, unless it is determined that the technology is expected to have an alternative future use. We record costs for certain development activities, such as preclinical studies and clinical trials, based on our evaluation of the progress to completion of specific tasks. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as applicable. Our recording of costs for certain development activities requires us to use estimates. We believe our estimates and assumptions are reasonable under the current conditions; however, actual results may differ from these estimates. Our research and development expenses are not currently tracked on a program-by-program basis for indirect and overhead costs. We use our personnel and infrastructure resources across multiple research and development programs directed toward identifying, developing, and commercializing product candidates.

Research and development expenses account for a significant portion of our operating expenses. We plan to incur research and development expenses for the foreseeable future as we expect to continue the development of our product candidates. We anticipate that our research and development expenses will be higher in fiscal year 2025 as compared to fiscal year 2024 due to an increase in clinical activities with respect to our product candidates as well as an increase in headcount.

At this time, due to the inherently unpredictable nature of preclinical and clinical developments as well as regulatory approval, we are unable to estimate with any certainty the costs we will incur and the timelines we will require in our continued development and commercialization efforts. As a result of these uncertainties, the successful development and completion of clinical trials as well as the regulatory approval process are uncertain and may not result in approved and commercialized products. Completion dates and completion costs can vary significantly for each product candidate and are difficult to predict. We will continue to make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to our ability to enter into partnerships with respect to each product candidate and the scientific and clinical success of each product candidate as well as ongoing assessments as to the commercial potential of each product candidate.

General and administrative expense

General and administrative expense consists primarily of personnel expenses, including salaries, benefits, insurance, and stock-based compensation expense, for employees in executive, accounting, commercialization, human resources, and other administrative functions. General and administrative expense also includes expenses related to pre-commercial activities, corporate facility costs, such as allocated rent and utilities, insurance premiums, legal fees related to corporate matters, and fees for auditing, accounting, and other consulting services.

We anticipate that our general and administrative expenses will increase in fiscal year 2025 as compared to fiscal year 2024 due to an increase in headcount.

Results of Operations

The following table summarizes the results of our operations for the years ended December 31, 2024 and 2023 (in thousands):

	Year ended December 31,		
	2024	2023	Change
Collaborative arrangement revenue	$ 4,055	$ 6,036	$ (1,981)
Total Revenue	4,055	6,036	(1,981)
Operating expenses			
Research and development	32,126	39,573	(7,447)
General and administrative	26,686	31,994	(5,308)
Total operating expenses	58,812	71,567	(12,755)
Loss from operations	(54,757)	(65,531)	10,774
Other income (expense), net	703	2,453	(1,750)
Net loss	$ (54,054)	$ (63,078)	$ 9,024

We believe the following table provides more transparency as to the type of research and development expenses incurred. The following table summarizes our research and development expenses by product candidate for the years ended December 31, 2024 and 2023 (in thousands):

	Year ended December 31,		
	2024	2023	Change
OCU400	$ 6,846	$ 7,318	$ (472)
OCU410 and OCU410ST	3,653	4,029	(376)
NeoCart	489	1,336	(847)
COVAXIN	25	8,824	(8,799)
Inhaled mucosal vaccine platform	2,464	629	1,835
OCU200	379	713	(334)
Unallocated costs:			
Research and development personnel costs	12,992	13,868	(876)
Facilities and other support costs	2,984	1,507	1,477
Other	2,294	1,349	945
Total research and development	$ 32,126	$ 39,573	$ (7,447)

Collaborative arrangement revenue

Collaborative arrangement revenue decreased by $2.0 million for the year ended December 31, 2024 compared to the year ended December 31, 2023. The decrease was due to the amount of co-development services provided by us to the business partner in the collaboration agreement.

Research and development expense

Research and development expense decreased by $7.4 million for the year ended December 31, 2024 compared to the year ended December 31, 2023. The decrease was primarily due to $8.8 million related to the termination of the COVAXIN program. This decrease was partially offset by an increase of $1.8 million related to OCU500, which is driven by an increase in preclinical activities and GMP manufacturing of Phase 1 clinical trial material.

General and administrative expense

General and administrative expense decreased by $5.3 million for the year ended December 31, 2024 compared to the year ended December 31, 2023. The decrease was primarily due to $2.7 million in professional service fees and $2.4 million related to reduced headcount.

Other income (expense), net

Other income (expense), net decreased by $1.8 million for the year ended December 31, 2024 compared to the year ended December 31, 2023. The decrease was primarily due to $1.1 million in interest earned on our cash and restricted cash, which had a lower average balance compared to prior year.

Liquidity and Capital Resources

As of December 31, 2024, we had $58.5 million in cash. We have not generated revenue from our product candidates to date, and have primarily funded our operations to date through the sale of common stock, warrants to purchase common stock, the issuance of convertible notes and debt, and grant proceeds. Since our inception and through December 31, 2024, we have raised an aggregate of $368.9 million to fund our operations, of which $325.1 million is from gross proceeds from the sale of our common stock and warrants, $10.3 million is from the issuance of convertible notes, $33.3 million is from the issuance of debt, and $0.2 million is from grant proceeds.

In November 2024, we entered into a debt financing transaction with Avenue for net proceeds of $29.2 million. The Loan and Security Agreement provides for term loans in an aggregate principal amount of up to $30.0 million delivered on November 6, 2024 (the "Term Loans"). The loan has a maturity date of November 1, 2028, of which the first 24 months are interest only, and bears interest at a variable rate per annum equal to the greater of the prime rate as reported in The Wall Street Journal plus 4.25% or 12.25%. Additionally, the Lenders have the right to convert an aggregate amount of up to $6.0 million of the outstanding principal amount into shares of our common stock at a conversion price per share equal to a 80% of the trading price on the date of conversion, which shall be at Lenders' option. In the event we elect to prepay the Term Loans in full, Lenders shall have 10 days to elect to exercise its conversion right prior to such prepayment. All conversion rights shall terminate on Term Loans payoff. In connection with the entry into the Loan and Security Agreement, we entered into a Subscription Agreement (the "Subscription Agreement") by and among us and the Lenders, pursuant to which we issued (i) 211,268 shares of common stock to Avenue 1 and (ii) 845,070 shares of common stock to Avenue 2, with an issue date as of November 6, 2024 (the "Equity Grant"). Notwithstanding the foregoing, the aggregate amount of our common stock issued pursuant to this conversion right and the Equity Grant shall not exceed a number of shares equal to 19.9% of our outstanding common stock. The agreement is collateralized by all of our assets in which the Agent is granted senior secured lien. We also granted the Lenders a negative pledge on our intellectual property.

During the year ended December 31, 2024, we issued and sold 32.7 million shares of our common stock at a public offering price of $1.15 per share pursuant to the July 2024 Public Offering (as defined below). We received net proceeds of $34.7 million after deducting equity issuance costs.

During the year ended December 31, 2023, we issued and sold 30.0 million shares of our common stock at a public offering price of $0.50 per share pursuant to an underwriting agreement (the "May 2023 Public Offering"). We received net proceeds of $14.8 million after deducting equity issuance costs.

During the year ended December 31, 2023, we sold 4.5 million shares of our common stock under the At Market Issuance Sales Agreement ("the Sales Agreement") with certain agents and received net proceeds of $5.6 million after deducting equity issuance costs of $0.2 million. The Sales Agreement was terminated in February 2023.

Since our inception, we have devoted substantial resources to research and development and have incurred significant net losses and may continue to incur net losses in the future. We incurred net losses of approximately $54.1 million and $63.1 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $340.2 million. In addition, as of December 31, 2024, we had accounts payable and accrued expenses and other current liabilities of $19.7 million, lease liability of $3.8 million, and indebtedness of $28.7 million.

The following table shows a summary of our cash flows for the year ended December 31, 2024 and the year ended December 31, 2023 (in thousands):

	Year ended December 31,	
	2024	2023
Net cash used in operating activities	$ (42,142)	$ (62,054)
Net cash (used in) provided by investing activities	(3,385)	3,077
Net cash provided by financing activities	64,858	20,881
Effect of changes in exchange rate on cash and restricted cash	28	(5)
Net (decrease) in cash and restricted cash	$ 19,359	$ (38,101)

Operating activities

Cash used in operating activities was $42.1 million for the year ended December 31, 2024, and primarily consisted of a net loss of $54.1 million adjusted for non-cash items including stock-based compensation of $7.4 million, depreciation and amortization of $2.0 million, non-cash lease expense of $0.9 million, non-cash expense from collaborative arrangements, net of $2.2 million, non-cash interest expense of $0.1 million, and a change in net working capital of $3.7 million.

Cash used in operating activities was $62.1 million for the year ended December 31, 2023, and primarily consisted of a net loss of $63.1 million adjusted for non-cash items including stock-based compensation of $9.2 million, impairment of advance for COVAXIN supply of $4.1 million, depreciation and amortization of $0.7 million, non-cash lease expense of $0.5 million, other non-cash items of $0.4 million, and a change in net working capital of $13.1 million.

Investing activities

Cash used in investing activities was $3.4 million for the year ended December 31, 2024, and primarily consisted of payments related to purchases of property and equipment. Cash provided by investing activities was $3.1 million for the year ended December 31, 2023, and primarily consisted of gross proceeds of $17.5 million from the maturities of marketable securities, classified as available-for-sale, during the year ended December 31, 2023, which was partially offset by purchases of $3.9 million of marketable securities, classified as available-for-sale, during the year ended December 31, 2023 as well as purchases of $10.5 million of property and equipment during the year ended December 31, 2023.

Financing activities

Cash provided by financing activities was $64.9 million for the year ended December 31, 2024 compared to $20.9 million for the year ended December 31, 2023. During the year ended December 31, 2024, cash provided by financing activities primarily consisted of gross proceeds of $37.6 million received from the July 2024 Public Offering before deducting equity issuance costs of $2.9 million as well as gross proceeds of $30.0 million received from the debt financing transaction with Avenue before deducting equity issuance costs of $0.8 million. During the year ended December 31, 2023, cash provided by financing activities primarily consisted of gross proceeds of a combined $20.8 million received from the May 2023 Public Offering and pursuant to the Sales Agreement.

Contractual Obligations

Licensing and Development Agreements

We have obligations under certain license and development agreements for our product candidates including annual payments, payments upon the achievement of certain milestones, and royalty payments based on net sales of licensed products. See Note 3 in the notes to the consolidated financial statements included in elsewhere in this Annual Report for information regarding our obligations under licensing and development agreements.

Lease Obligations

We have obligations under our operating leases, which include leased office, laboratory, and future manufacturing space, located in Malvern, Pennsylvania and other locations. As of December 31, 2024, we had future minimum operating lease base rent payment obligations of $5.2 million, with $0.9 million payable within 12 months of December 31, 2024. See Note 6 in the

notes to the consolidated financial statements included elsewhere in this Annual Report for information regarding our obligations under lease obligations.

Indebtedness

We have outstanding debt related to the funds borrowed from EB5 Life Sciences pursuant to the United States government's Immigrant Investor Program, commonly known as the EB-5 program. Pursuant to the loan agreement entered into with EB-5 Life Sciences, we have borrowed $2.5 million to date. We also entered into the Loan and Security Agreement with Avenue that is secured by a lien on all of our assets. Pursuant to the Loan and Security Agreement entered into with Avenue, we have borrowed $30.0 million to date. See Note 8 in the notes to the consolidated financial statements included elsewhere in this Annual Report for information regarding our obligations under the EB-5 loan agreement and the Loan and Security Agreement.

Funding requirements

We expect to continue to incur significant expenses in connection with our ongoing activities, particularly as we continue research and development, including preclinical and clinical development of our product candidates, prepare to manufacture our product candidates, prepare for the potential commercialization of our product candidates, add operational, financial, and information systems to execute our business plan, maintain, expand, and protect our patent portfolio, explore strategic licensing, acquisition, and collaboration opportunities to expand our product candidate pipeline to support our future growth; expand headcount to support our development, commercialization, and business efforts, and operate as a public company.

Factors impacting our future funding requirements include, without limitation, the following:

- the initiation, progress, timing, costs, and results of trials for our product candidates;
- the preparation and submission of Investigational New Drug applications, or INDs, with the FDA for current and future product candidates;
- the outcome, timing, and cost of the regulatory approval process for our product candidates;
- the costs of manufacturing and commercialization;
- the costs related to doing business internationally with respect to the development and commercialization of our product candidates;
- the cost of filing, prosecuting, defending, and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us;
- the acquisition of or in-licensing of additional product candidates and technologies;
- the costs of expanding infrastructure to support our development, commercialization, and business efforts, including the costs related to the development of a laboratory and manufacturing facility;
- the costs involved in recruiting and retaining skilled personnel;
- the extent to which we in-license or acquire other products, product candidates, or technologies and out-license our product candidates; and
- the impact of geopolitical turmoil, macroeconomic conditions, social unrest, political instability, terrorism, or other acts of war.

As of December 31, 2024, we had cash of approximately $58.5 million. This amount will not meet our capital requirements over the next 12 months. We believe that our cash and cash equivalents will enable us to fund our operations into the first quarter of 2026. Due to the inherent uncertainty involved in making estimates and the risks associated with the research, development, and commercialization of biotechnology products, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us.

We are subject to risks and uncertainties frequently encountered by companies in the biotech industry, and while we intend to continue research, development, and commercialization efforts for our product candidates, we will require significant additional funding. If we are unable to obtain additional funding in the future and/or our research, development, and commercialization efforts require higher than anticipated capital, there will be a negative impact on our financial viability. We will continue to explore options to fund our operations through public and private placements of equity and/or debt, payments from potential strategic research and development arrangements, sales of assets, licensing and/or collaboration arrangements with

pharmaceutical companies or other institutions, funding from the government, particularly for the development of our novel inhaled mucosal vaccine platform, or funding from other third parties. Such financing and funding may not be available at all, or on terms that are favorable to us. While management believes that we have a plan to fund operations, our plan may not be successfully implemented. If we cannot obtain the necessary funding, we will need to delay, scale back, or eliminate some or all of our research and development programs and commercialization efforts; consider other various strategic alternatives, including a merger or sale; or cease operations. If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition, and results of operations could be materially adversely affected.

As a result of these factors, together with the anticipated continued spending that will be necessary to continue to research, develop, and commercialize our product candidates, there is substantial doubt about our ability to continue as a going concern within one year after the date that these consolidated financial statements are issued. The consolidated financial statements do not contain any adjustments that might result from the resolution of any of the above uncertainties.

Off-Balance Sheet Arrangements

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

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States ("GAAP"). The preparation of our financial statements requires us to make estimates and judgments that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reported period. We base our estimates on historical experience, known trends and events, and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

While our significant accounting policies are described in more detail in the notes to the consolidated financial statements included elsewhere in this Annual Report, we believe that the following accounting policies and estimates are those most critical to the preparation of our consolidated financial statements:

Research and Development and Clinical Trial Accruals

As part of the process of preparing the consolidated financial statements included elsewhere in this Annual Report, we are required to estimate and record expenses, for which a large portion are research and development expenses. Research and development expenses include, among other categories, development, clinical trials, patent costs, and regulatory compliance costs incurred with research organizations, contract manufacturers, and other third-party vendors. The estimation process involves identifying services that have been performed on our behalf by third-parties, estimating and accruing expenses in our consolidated financial statements based on the evaluation of the progress to completion of specific tasks and the facts and circumstances known to us at the time of the estimate, and assessing the accuracy of these estimates going forward to determine if adjustments are required. We periodically collaborate with our third-party vendors to assist in determining our estimates. Payments for these activities performed by our third-party vendors are based on the terms of the individual arrangements with our third-party vendors, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as applicable. We believe our estimates and assumptions are reasonable under the current conditions; however, actual results may differ from these estimates. Any changes to estimates will be recorded in the period in which a circumstance causing a change in estimate becomes known and the impact of any change in estimate could be material.

Collaborative Arrangements and Revenue Recognition

We analyze our collaborative arrangements to assess whether they are within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808") to determine 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. This assessment is performed throughout the life of the arrangements based on changes to the arrangements. For collaborative arrangements within the scope of ASC 808 we may analogize to ASC 606 for certain elements.

We identify the goods or services promised within each collaborative arrangement and assesses whether each promised good or service is distinct for the purpose of identifying the performance obligations in the contract. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that: (i) the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer and (ii) the entity's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

The allocation of the transaction price to the performance obligations in proportion to their standalone selling prices is determined at contract inception. If the consideration promised in a contract includes a variable amount, we estimate the amount of consideration to which it will be entitled in exchange for transferring the promised goods or services to a customer. We determine the amount of variable consideration by using the expected value method or the most likely amount method. We include the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price is the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, we re-evaluate the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the period of adjustment.

In determining the transaction price, we adjust consideration for the effects of the time value of money if the timing of payments provides us with a significant benefit of financing. We do not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the counterparty and the transfer of the promised goods or services to the counterparty will be one year or less. We assess its collaboration arrangements in order to determine whether a significant financing component exists and concluded that a significant financing component does not exist in any of its arrangements.

We recognize as collaboration revenue the amount of the transaction price that is allocated to the respective performance obligation as each performance obligation is satisfied over time, with progress toward completion measured based on actual costs incurred relative to total estimated costs to be incurred over the life of the arrangement. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which we our expected to complete their performance obligations under the arrangements and in determining the estimated market value of the co-development services included in the transaction price. We evaluate the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. Adjustments to original estimates will be required as work progresses and additional information becomes known, even though the scope of the work required under the contract may not change. Any adjustment as a result of a change in estimates is made when facts develop, events become known, or an adjustment is otherwise warranted.

Under our collaborative arrangements, the timing of revenue recognition and receipt of consideration may differ, and result in assets and liabilities. Assets represent revenues recognized in excess of the consideration received under collaborative arrangement. Liabilities represent the consideration received in excess of revenues recognized under collaborative arrangement.

Stock-based compensation

We account for our stock-based compensation awards in accordance with the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 718, *Compensation—Stock Compensation* ("ASC 718"). We have issued stock-based compensation awards including stock options and restricted stock units ("RSUs"), and market-condition based restricted stock units ("PSUs"), and we also account for certain issuances of preferred stock and warrants in accordance with ASC 718. ASC 718 requires all stock-based payments, including grants of stock options and RSUs, and PSUs, to be recognized in the consolidated statements of operations and comprehensive loss based on their grant date fair values. We use the Black-Scholes option-pricing model to determine the fair value of options granted. For RSUs, the fair value of the RSUs is determined by the market price of a share of our common stock on the grant date. For PSUs, we determine fair value by using a Monte Carlo simulation technique. We recognize forfeitures as they occur.

Expense related to stock-based compensation awards granted with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Stock-based awards generally vest over a one to three year requisite service period. Stock options have a contractual term of 10 years. Expense for stock-based compensation awards with performance-based vesting conditions is only recognized when the performance-based vesting condition is deemed probable to occur. Expense for stock-based compensation awards with market-based and service-based vesting conditions is recognized ratably over the grantee's requisite service period. Compensation cost is not adjusted based on the actual achievement of the market-based performance goals. Expense related to stock-based compensation awards are recorded to research and development expense or general and administrative expense based on the

underlying function of the individual that was granted the stock-based compensation award. Shares issued upon stock option exercise, PSU and RSU vesting are newly issued common shares.

Estimating the fair value of stock options requires the input of subjective assumptions, including the expected term of the stock option, stock price volatility, the risk-free interest rate, and expected dividends. Estimating the fair value of PSUs requires the input of subjective assumptions, including stock price volatility, total shareholder return ("TSR") ranking, the risk-free rate, and expected dividends. The assumptions used in our Black-Scholes option-pricing model and Monte Carlo simulation technique represent management's best estimates and involve a number of variables, uncertainties, assumptions, and the application of management's judgment, as they are inherently subjective. If any assumptions change, our stock-based compensation expense could be materially different in the future.

The assumptions used in our Black-Scholes option-pricing model for stock options and in our Monte Carlo simulation technique for PSUs are as follows, unless noted otherwise:

Expected Term. As we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term, the expected term of employee stock options subject to service-based vesting conditions is determined using the "simplified" method, as prescribed in SEC's Staff Accounting Bulletin No. 107, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the option. This expected term assumption is not an assumption used in our Monte Carlo simulation technique for PSUs. The expected term of the PSUs is equal to the performance period of the PSUs.

Expected Volatility. The expected volatility is based on historical volatilities of Ocugen and similar entities within Ocugen's industry for periods commensurate with the assumed expected term.

Risk-Free Interest Rate. The risk-free interest rate is based on the interest rate payable on United States Treasury securities in effect at the time of grant for a period that is commensurate with the assumed expected term.

Expected Dividends. The expected dividend yield is 0% because we have not historically paid, and do not expect for the foreseeable future to pay, a dividend on our common stock.

TSR ranking. The TSR, over a three-year period, is relative to the TSR, for that same period, as related to other companies within the Nasdaq Biotechnology Index. This assumption is only used for the market-based PSUs.

Stock-based compensation expense was $7.4 million and $9.2 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had $5.4 million of unrecognized stock-based compensation expense, which is expected to be recognized over a remaining weighted-average period of 1.6 years.

Recent Accounting Pronouncements

For a discussion of recent accounting pronouncements, see Note 2 in the notes to the consolidated financial statements included elsewhere in this Annual Report.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk.

Not applicable.

Item 8. Financial Statements and Supplementary Data

The financial statements required by this item are set forth beginning on page F-1 of this report and are incorporated herein by reference. The report of our Independent Registered Public Accounting Firm, PricewaterhouseCoopers LLP, Public Company Accounting Oversight Board identification number 238, is included therein. The report of our previous Independent Registered Public Accounting Firm, Ernst & Young LLP, Public Company Accounting Oversight Board identification number 42, is also included therein.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of December 31, 2024. Based upon that evaluation, our principal executive officer and principal financial officer concluded that, as of December 31, 2024, the end of the period covered by this Annual Report, our disclosure controls and procedures are effective in providing reasonable assurance that (a) the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and (b) such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

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 Rule 13a-15(f) and 15d-15(f) under the Exchange Act as a process designed by, or under the supervision of, our principal executive officer and principal financial officer 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 our financial statements for external purposes in accordance with GAAP 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 GAAP, 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 our consolidated financial statements.

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

Under the supervision of and with the participation of our principal executive officer and principal financial officer, our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2024 based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in *Internal Control-Integrated Framework* (2013). Based on this assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2024.

This Annual Report does not include an attestation report on internal control over financial reporting by our independent registered public accounting firm since we are a smaller reporting company under the rules of the SEC.

Remediation

As previously disclosed, in our Annual Report for the year ended December 31, 2023, and in our Quarterly Reports on Form 10-Q for the periods ended March 31, June 30 and September 30, 2024, during the preparation of our consolidated financial statements for the year ended December 31, 2023, management identified a material weakness in our internal control over financial reporting. We did not design and maintain effective controls over the accounting for collaborative arrangements.

Since identifying the material weakness, management has designed and implemented controls to obtain relevant information from third parties on a timely basis, including actual costs incurred, actual noncash consideration received, and estimates to complete and review the accounting for collaborative arrangements during the financial statement close process.

During the quarter ended December 31, 2024, our management completed testing of the remediation activities and determined that the newly implemented controls had been operating effectively for a sufficient period to conclude that the previously identified material weakness was remediated.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting during our most recent fiscal quarter ended December 31, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

Insider Trading Arrangements

During our three months ended December 31, 2024, no director or officer adopted or terminated any Rule 10b5-1 trading arrangement, and/or any non-Rule 10b5-1 trading arrangement (as such terms are defined pursuant to Item 408(a) of Regulation S-K).

Item 9C. Disclosure Regarding Foreign Jurisdiction that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this Item is incorporated by reference from the discussion responsive thereto contained in the Proxy Statement for our 2025 Annual Meeting of Stockholders or in an amendment on Form 10-K/A, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report relates.

Item 11. Executive Compensation.

The information required by this Item (excluding the information under the heading "Pay Versus Performance") is incorporated by reference from the discussion responsive thereto contained in the Proxy Statement for our 2025 Annual Meeting of Stockholders or in an amendment on Form 10-K/A, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report relates.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this Item is incorporated by reference from the discussion responsive thereto contained in the Proxy Statement for our 2025 Annual Meeting of Stockholders or in an amendment on Form 10-K/A, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report relates.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this Item is incorporated by reference from the discussion responsive thereto contained in the Proxy Statement for our 2025 Annual Meeting of Stockholders or in an amendment on Form 10-K/A, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report relates.

Item 14. Principal Accountant Fees and Services.

The information required by this Item is incorporated by reference from the discussion responsive thereto contained in the Proxy Statement for our 2025 Annual Meeting of Stockholders or in an amendment on Form 10-K/A, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

PART IV

Item 15. Exhibit and Financial Statement Schedules.

The financial statements, financial statement schedules, and exhibits filed as part of this Annual Report are as follows:

(a)(1) Financial Statements

See "Index to Consolidated Financial Statements" beginning on page F-1 of this report.

(a)(2) Financial Statement Schedules

Not applicable.

(a)(3) Exhibits

The exhibits required to be filed as part of this Annual Report are listed in the Exhibit Index attached hereto and are incorporated herein by reference.

EXHIBIT INDEX

Exhibit	Description
3.1	Sixth Amended and Restated Certificate of Incorporation (filed as Exhibit 3.1 to the Registrant's Current Report on Form 8-K as filed on December 8, 2014, and incorporated herein by reference)
3.2	Amendment to Sixth Amended and Restated Certificate of Incorporation related to the Reverse Stock Split and the Authorized Share Increase (filed as Exhibit 3.1 to the Registrant's Current Report on Form 8-K as filed October 1, 2019, and incorporated herein by reference)
3.3	Amendment to Sixth Amended and Restated Certificate of Incorporation related to the Name Change (filed as Exhibit 3.2 to the Registrant's Current Report on Form 8-K as filed on October 1, 2019, and incorporated herein by reference)
3.4	Amendment to Sixth Amended and Restated Certificate of Incorporation related to the Increase in Authorized Shares of Common Stock (filed as Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q as filed on May 7, 2021, and incorporated herein by reference)
3.5	Amendment to Sixth Amended and Restated Certificate of Incorporation related to the Authorized Share Increase (filed as Exhibit 3.2 to the Registrant's Quarterly Report on Form 10-Q as filed on August 8, 2024, and incorporated herein by reference)
3.6	Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Preferred Stock of Histogenics Corporation (filed as Exhibit 3.3 to the Registrant's Current Report on Form 8-K as filed on September 16, 2016, and incorporated herein by reference)
3.7	Certificate of Designation of Preferences, Rights and Limitations of Series B Convertible Preferred Stock of Ocugen, Inc. (filed as Exhibit 3.5 to the Registrant's Annual Report on Form 10-K filed on March 19, 2021 and incorporated herein by reference)
3.8	Certificate of Designation of Preferences, Rights and Limitations (filed as Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed on May 10, 2024, and incorporated herein by reference)
3.9	Second Amended and Restated Bylaws of Ocugen, Inc. (filed as Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q as filed on August 21, 2023, and incorporated herein by reference)
3.10	Amendment to Second Amended and Restated Bylaws of Ocugen, Inc. (filed as Exhibit 3.1 to the Registrant's Current Report of Form 8-K as filed on March 20, 2024, and incorporated herein by reference)
4.1	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934 (filed as Exhibit 4.1 to the Registrant's Annual Report on Form 10-K as filed on February 28, 2022, and incorporated herein by reference)
4.2	Form of Common Stock Purchase Warrant (filed as Exhibit 4.8 to the Registrant's Annual Report on Form 10-K filed on March 19, 2021 and incorporated herein by reference)
10.1+	Ocugen, Inc. 2014 Stock Option Plan (filed as Exhibit 10.30 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.2+	Form of Incentive Stock Option Agreement under Ocugen, Inc. 2014 Stock Option Plan (filed as Exhibit 10.31 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.3+	Form of Nonstatutory Stock Option Agreement under Ocugen, Inc. 2014 Stock Option Plan (filed as Exhibit 10.32 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.4+	Ocugen, Inc. 2019 Equity Incentive Plan (filed as Appendix A to the Registrant's Proxy Statement on Schedule 14A as filed on November 8, 2019, and incorporated herein by reference)
10.5+	Form of Incentive Stock Option Agreement under Ocugen, Inc. 2019 Equity Incentive Plan (filed as Exhibit 10.5 to the Registrant's Annual Report on Form 10-K as filed on February 28, 2023 and incorporated herein by reference)
10.6+	Form of Non-Qualified Stock Option Agreement under Ocugen, Inc. 2019 Equity Incentive Plan (filed as Exhibit 10.6 to the Registrant's Annual Report on Form 10-K as filed on February 28, 2023 and incorporated herein by reference)

10.7+	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement under Ocugen, Inc. 2019 Equity Incentive Plan (filed as Exhibit 10.7 to the Registrant's Annual Report on Form 10-K as filed on February 28, 2023 and incorporated herein by reference)
10.8+	Form of Performance-Vested Stock Option Agreement under Ocugen, Inc. 2019 Equity Incentive Plan (filed as Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q as filed on May 7, 2021, and incorporated herein by reference)
10.9+	Form of Non-Qualified Stock Option Agreement for Inducement Non-Qualified Stock Option Awards
10.10+	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement for Inducement Restricted Stock Unit Awards (filed as Exhibit 10.10 to the Registrant's Annual Report on Form 10-K as filed on February 28, 2023 and incorporated herein by reference)
10.11#	Exclusive License Agreement, effective as of March 3, 2014, between The Regents of the University of Colorado and Ocugen Opco, Inc. (filed as Exhibit 10.33 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)

10.12#	First Amendment to the Exclusive License Agreement, dated as of January 23, 2017, by and between The Regents of the University of Colorado and Ocugen Opco, Inc. (filed as Exhibit 10.34 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.13	Letter of Understanding, dated November 8, 2017, between The Regents of the University of Colorado and Ocugen Opco, Inc. (filed as Exhibit 10.35 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.14#	Exclusive License Agreement, effective as of December 19, 2017, between The Schepens Eye Research Institute, Inc. and Ocugen Opco, Inc. (filed as Exhibit 10.37 to the Registrant's Registration Statement on Form S-4 (SEC File No. 333-232147), as filed on June 14, 2019, and incorporated herein by reference)
10.15#	Co-Development, Supply and Commercialization Agreement, dated as of January 31, 2021, by and between the Registrant and Bharat Biotech International Limited (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on May 7, 2021, and incorporated herein by reference)
10.16	First Amendment to Co-Development, Supply and Commercialization Agreement, dated as of May 29, 2021, by and between the Registrant and Bharat Biotech International Limited (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on August 6, 2021, and incorporated herein by reference)
10.17#	Second Amendment to Co-Development, Supply and Commercialization Agreement, dated as of April 15, 2022, by and between the Registrant and Bharat Biotech International Limited (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on May 6, 2022, and incorporated herein by reference)
10.18#	Development and Commercial Supply Agreement, dated September 29, 2021, by and between the Registrant and Bharat Biotech International Limited (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on November 9, 2021, and incorporated herein by reference)
10.19#	Co-Development and Commercialization Agreement, dated as of September 27, 2019, by and among the Registrant and CanSino Biologics Inc. (filed as Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q as filed on November 12, 2019, and incorporated herein by reference)
10.20#	First Amendment to the Co-Development and Commercialization Agreement, dated September 30, 2021, by and between the Registrant and CanSino Biologics, Inc. (filed as Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q as filed on November 9, 2021, and incorporated herein by reference)
10.21	Second Amendment to the Co-Development and Commercialization Agreement, dated November 21, 2022, by and between the Registrant and CanSino Biologics, Inc.
10.22#	Third Amendment to the Co-Development and Commercialization Agreement, dated April 11, 2023, by and between the Registrant and CanSino Biologics, Inc.
10.23#	Exclusive License Agreement by and between the Registrant and The Washington University, dated as of September 23, 2022 (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on November 8, 2022, and incorporated herein by reference)
10.24#	First Amendment to the Exclusive License Agreement by and between the Registrant and The Washington University, dated as of January 31, 2023
10.25#	Second Amendment to the Exclusive License Agreement by and between the Registrant and The Washington University, dated as of November 28, 2023
10.26	Loan and Security Agreement, effective as of September 12, 2016, by and between EB5 Life Sciences, LP and Ocugen Opco, Inc. (filed as Exhibit 10.42 to the Registrant's Registration Statement on Form S-4/A (SEC File No. 333-232147), as filed on July 23, 2019, and incorporated herein by reference)
10.27+	Amended and Restated Employment Agreement, dated as of January 1, 2020, by and between the Registrant and Shankar Musunuri (filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K as filed on January 3, 2020, and incorporated herein by reference)

10.28+	First Amendment to Amended and Restated Executive Employment Agreement, dated as of April 27, 2022, by and between the Registrant and Shankar Musunuri (filed as Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q as filed on May 6, 2022, and incorporated herein by reference)
10.29+	Amended and Restated Executive Employment Agreement, dated as of December 16, 2021, by and between the Registrant and Arun Upadhyay
10.30+	First Amendment to Amended and Restated Executive Employment Agreement, dated as of August 26, 2022, by and between the Registrant and Arun Upadhyay
10.31†	Agreement dated as of June 22, 2012 between the Registrant and Purpose Co., Ltd. f/k/a Takagi Sangyo Co. Ltd. and f/k/a Takagi Industrial Co., Ltd. (filed as Exhibit 10.20 to the Registrant's Registration Statement on Form S-1 (SEC File No. 333-199202), as filed on October 7, 2014, and incorporated herein by reference)
10.35†	First Amendment to License Agreement, dated May 9, 2016, between the Registrant and Purpose Co., Ltd., f/k/a Takagi Sangyo Co. Ltd. (filed as Exhibit 10.33 to the Registrant's Quarterly Report on Form 10-Q as filed on August 11, 2016, and incorporated herein by reference)
10.33+	Executive Employment Agreement, dated as of March 18, 2024, by and between the Registrant and Huma Qamar (filed as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q as filed on May 14, 2024, and incorporated herein by reference)
10.36#	Loan and Security Agreement by and among Ocugen, Inc., Ocugen OpCo, Inc., Avenue Capital Management II, L.P., Avenue Venture Opportunities Fund II, L.P. and Avenue Venture Opportunities Fund, L.P., dated November 6, 2024 (filed as Exhibit 10.2 to the Registrant's Current Report on Form 8-K as filed on February 7, 2025, and incorporated herein by reference)
10.36+	Form of Performance Restricted Stock Unit Award Agreement
10.37#	Supplement to Loan and Security Agreement by and among Ocugen, Inc., Ocugen OpCo, Inc., Avenue Capital Management II, L.P., Avenue Venture Opportunities Fund II, L.P. and Avenue Venture Opportunities Fund, L.P., dated November 6, 2024 (filed as Exhibit 10.3 to the Registrant's Current Report on Form 8-K as filed on February 7, 2025, and incorporated herein by reference)
10.38#	Subscription Agreement by and among Ocugen, Inc., Ocugen OpCo, Inc., Avenue Capital Management II, L.P., Avenue Venture Opportunities Fund II, L.P. and Avenue Venture Opportunities Fund, L.P., dated November 6, 2024 (filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K as filed on February 7, 2025, and incorporated herein by reference)
10.38*	Executive Employment Agreement, dated as of September 9, 2024, by and between the Registrant and Ramesh Ramachandran
19.1*	Insider Trading Policy
21.1	List of Subsidiaries
23.1*	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm to the Registrant
23.2*	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm to the Registrant
31.1*	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of the Chief Financial Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350
97+*	Compensation Recovery Policy (filed as Exhibit 97 to the Registrant's Annual Report on Form 10-K as filed April 16, 2024, and incorporated herein by reference)
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
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	The cover page from this Annual Report on Form 10-K, formatted in Inline XBRL with applicable taxonomy extension information contained in Exhibits 101

_____

* Filed herewith.

** Furnished herewith.

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

+ Indicates a management contract or compensatory plan or arrangement.

† Confidential treatment has been granted with respect to certain portions of this exhibit.

Item 16. 10-K Summary

Not applicable.

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.

Ocugen, Inc.

Dated: March 5, 2025

/s/ Shankar Musunuri

Shankar Musunuri, Ph.D., MBA
Chairman, Chief Executive Officer & Co-Founder
(Principal Executive Officer)

Dated: March 5, 2025

/s/ Ramesh Ramachandran

Ramesh Ramachandran, CPA, MBA, CMA
Chief Accounting Officer
(Principal Financial Officer and Principal Accounting Officer)

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Shankar Musunuri Shankar Musunuri	Chairman, Chief Executive Officer & Co-Founder (Principal Executive Officer)	March 5, 2025
/s/ Ramesh Ramachandran Ramesh Ramachandran	Chief Accounting Officer (Principal Financial Officer and Principal Accounting Officer)	March 5, 2025
/s/ Junge Zhang Junge Zhang	Director	March 5, 2025
/s/ Uday Kompella Uday Kompella	Director	March 5, 2025
/s/ Kirsten Castillo Kirsten Castillo	Director	March 5, 2025
/s/ Prabhavathi Fernandes Prabhavathi Fernandes	Director	March 5, 2025
/s/ Mama Whittington Mama Whittington	Director	March 5, 2025

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OCUGEN, INC.

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Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Ocugen, Inc.

Opinion on the Financial Statements

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

Substantial Doubt about the Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has incurred recurring net losses since inception that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated 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 audit of these consolidated financial statements 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 consolidated 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 audit 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 audit included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audit provides a reasonable basis for our opinion.

Critical Audit Matters

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

Revenue Recognition - Collaborative Arrangement Revenue

As described in Notes 2 and 3 to the consolidated financial statements, the Company recorded collaborative arrangement revenue of $4.1 million for the year ended December 31, 2024. The Company recognizes collaborative arrangement revenue over time using an input method using ratio of costs incurred to date compared to total estimated costs required to satisfy the performance obligation. The Company recognizes as collaboration revenue the amount of the transaction price that is allocated to the respective performance obligation as each performance obligation is satisfied over time, with progress toward completion measured based on actual costs incurred relative to total estimated costs to be incurred over the life of the arrangement.

The principal consideration for our determination that performing procedures relating to revenue recognition for collaborative arrangement revenue is a critical audit matter is a high degree of auditor effort in performing procedures related to collaborative

arrangement revenue. As previously disclosed by management, a material weakness existed during the year related to this matter.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included, among others (i) reading the collaborative arrangement; (ii) evaluating the reasonableness of the methodology applied by management; and (iii) testing the completeness and accuracy of the actual costs incurred to date, including by confirming costs incurred by the collaboration partner directly with the collaboration partner.

/s/ PricewaterhouseCoopers LLP

Philadelphia, Pennsylvania

March 5, 2025

We have served as the Company's auditor since 2024.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Ocugen, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheet of Ocugen, Inc. (the Company) as of December 31, 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for the year then ended, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023, and the results of its operations and its cash flows for the year then ended in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

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 audit 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 audit 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 audit 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 audit 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 audit provides a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We served as the Company's auditor from 2018 to 2024.

Philadelphia, Pennsylvania

April 16, 2024

OCUGEN, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share amounts)

	As of December 31,	
	2024	2023
Assets		
Current assets		
Cash	$ 58,514	$ 39,462
Prepaid expenses and other current assets	3,168	3,509
Total current assets	61,682	42,971
Property and equipment, net	16,554	17,290
Restricted cash	307	—
Other assets	3,899	4,286
Total assets	$ 82,442	$ 64,547
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 4,243	$ 3,172
Accrued expenses and other current liabilities	15,500	13,343
Operating lease obligations	519	574
Current portion of long term debt	1,326	—
Total current liabilities	21,588	17,089
Non-current liabilities		
Operating lease obligations, less current portion	3,313	3,567
Long term debt, net	27,345	2,800
Other non-current liabilities	564	527
Total non-current liabilities	31,222	6,894
Total liabilities	52,810	23,983
Commitments and contingencies (Note 14)		
Stockholders' equity		
Preferred stock; $0.01 par value; 10,000,000 shares authorized at December 31, 2024 and 2023		
Series B Convertible Preferred Stock; zero shares issued and outstanding at December 31, 2024 and 54,745 issued and outstanding at December 31, 2023	—	1
Common stock; $0.01 par value; 390,000,000 shares authorized; 291,489,058 and 256,688,304 shares issued, and 291,367,558 and 256,566,804 shares outstanding at December 31, 2024 and 2023, respectively	2,915	2,567
Treasury stock, at cost, 121,500 shares at December 31, 2024 and 2023	(48)	(48)
Additional paid-in capital	366,938	324,191
Accumulated other comprehensive income	48	20
Accumulated deficit	(340,221)	(286,167)
Total stockholders' equity	29,632	40,564
Total liabilities and stockholders' equity	$ 82,442	$ 64,547

See accompanying notes to consolidated financial statements.

OCUGEN, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)

	Year ended December 31,	
	2024	2023
Collaborative arrangement revenue	$ 4,055	$ 6,036
Total revenue	4,055	6,036
Operating expenses		
Research and development	32,126	39,573
General and administrative	26,686	31,994
Total operating expenses	58,812	71,567
Loss from operations	(54,757)	(65,531)
Other income (expense), net	703	2,453
Net loss	$ (54,054)	$ (63,078)
Other comprehensive income (loss)		
Foreign currency translation adjustment	28	(5)
Unrealized gain (loss) on marketable securities	—	(1)
Comprehensive loss	$ (54,026)	$ (63,084)
Net loss attributable to common shareholders— basic and diluted	(54,010)	(63,078)
Weighted shares used in calculating net loss per common share — basic and diluted	270,995,121	244,327,057
Net loss per share attributable to common shareholders — basic and diluted	$ (0.20)	$ (0.26)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	(44)	—
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	54,745	—
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	$ (0.80)	$ —

See accompanying notes to consolidated financial statements.

OCUGEN, INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(in thousands, except share amounts)

	Series B Convertible Preferred Stock		Common Stock		Treasury Stock	Additional Paid in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount					
Balance at December 31, 2022	54,745	$ 1	221,721,182	$ 2,217	$ (48)	$ 294,874	$ 26	$ (223,089)	$ 73,981
Stock-based compensation expense	—	—	—	—	—	9,217	—	—	9,217
Issuance of common stock for stock option exercises and restricted stock unit vesting, net	—	—	488,166	5	—	119	—	—	124
Issuance of common stock for capital raises, net	—	—	34,478,956	345	—	19,981	—	—	20,326
Other comprehensive income (loss)	—	—	—	—	—	—	(6)	—	(6)
Net loss	—	—	—	—	—	—	—	(63,078)	(63,078)
Balance at December 31, 2023	54,745	$ 1	256,688,304	$ 2,567	$ (48)	$ 324,191	$ 20	$ (286,167)	$ 40,564
Stock-based compensation expense	—	—	—	—	—	7,427	—	—	7,427
Issuance of common stock for stock option exercises and restricted stock unit vesting, net	—	—	1,027,025	10	—	(80)	—	—	(70)
Issuance of Common Stock for capital raises, net	—	—	33,773,729	338	—	35,399	—	—	35,737
Series B Convertible Preferred Stock reacquisition	(54,745)	(1)	—	—	—	1	—	—	—
Other comprehensive income	—	—	—	—	—	—	28	—	28
Net loss	—	—	—	—	—	—	—	(54,054)	(54,054)
Balance at December 31, 2024	—	$ —	291,489,058	$ 2,915	$ (48)	$ 366,938	$ 48	$ (340,221)	$ 29,632

See accompanying notes to consolidated financial statements.

OCUGEN, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year ended December 31,	
	2024	2023
Cash flows from operating activities		
Net loss	$ (54,054)	$ (63,078)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	1,968	704
Amortization (accretion) on marketable securities	—	(182)
Non-cash interest expense	116	116
Non-cash lease expense	856	538
Non-cash income from collaborative arrangements, net	(2,157)	(696)
Stock-based compensation expense	7,427	9,217
Impairment of advance for COVAXIN supply	—	4,074
Loss on disposal of fixed assets related to COVAXIN	—	363
Other	37	46
Changes in assets and liabilities:		
Prepaid expenses and other current assets	361	(296)
Accounts payable and accrued expenses	4,137	(12,343)
Lease obligations	(833)	(517)
Net cash used in operating activities	(42,142)	(62,054)
Cash flows from investing activities		
Purchases of marketable securities	—	(3,947)
Proceeds from the maturities of marketable securities	—	17,500
Purchases of property and equipment	(3,385)	(10,476)
Repayment of note receivable	—	—
Net cash (used in) provided by investing activities	(3,385)	3,077
Cash flows from financing activities		
Proceeds (payments) from issuance of common stock, net	38,556	20,804
Payment of equity issuance costs	(2,889)	(355)
Proceeds from issuance of debt	30,000	500
Payments of debt issuance costs	(809)	(68)
Net cash provided by financing activities	64,858	20,881
Effect of changes in exchange rate on cash and restricted cash	28	(5)
Net (decrease) in cash and restricted cash	19,359	(38,101)
Cash and restricted cash at beginning of period	39,462	77,563
Cash and restricted cash at end of period	$ 58,821	$ 39,462

OCUGEN, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS (CONTINUED)
(in thousands)

	Year ended December 31,	
	2024	2023
Supplemental disclosure of non-cash investing and financing transactions:		
Purchase of property and equipment	$ —	$ 2,189
Right-of-use assets related to operating leases	$ 103	$ 572
Series B Convertible Preferred Stock reacquisition	$ (1)	$ —
Issuance of common stock for Avenue Capital agreement	$ 1,000	$ —

See accompanying notes to consolidated financial statements.

OCUGEN, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of Business

Ocugen, Inc., together with its wholly owned subsidiaries ("Ocugen" or the "Company"), is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics and vaccines that improve health and offer hope for patients across the globe. The Company is headquartered in Malvern, Pennsylvania, and manages its business as one operating segment. Refer to Note 15 for additional information.

Going Concern

The Company has incurred recurring net losses since inception and has funded its operations to date through the sale of common stock, warrants to purchase common stock, the issuance of convertible notes and debt, and grant proceeds. The Company incurred net losses of approximately $54.1 million and $63.1 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, the Company had an accumulated deficit of $340.2 million and cash totaling $58.5 million. This amount will not be sufficient to fund the Company's operations over the next 12 months after the date that the consolidated financial statements are issued. Due to the inherent uncertainty involved in making estimates and the risks associated with the research, development, and commercialization of biotechnology products, the Company may have based this estimate on assumptions that may prove to be different than actuals, and the Company's operating plan may change as a result of many factors currently unknown to the Company.

In November 2024, the Company entered into a debt financing transaction with Avenue Capital for net proceeds of $29.2 million, refer to Note 8 for additional information. Despite this additional funding, the Company is subject to risks and uncertainties frequently encountered by companies in its industry, and while the Company intends to continue its research, development, and commercialization efforts for its product candidates, the Company will require significant additional funding. If the Company is unable to obtain additional funding in the future and/or its research, development, and commercialization efforts require higher than anticipated capital, there will be a negative impact on the financial viability of the Company. The Company will continue to explore options to fund its operations through public and private placements of equity and/or debt, payments from potential strategic research and development arrangements, sales of assets, licensing and/or collaboration arrangements with pharmaceutical companies or other institutions, funding from the government, particularly for the development of the Company's novel inhaled mucosal vaccine platform, or funding from other third parties. Such financing and funding may not be available at all, or on terms that are favorable to the Company. While Company management believes that it has a plan to fund operations, its plan may not be successfully implemented. If we cannot obtain the necessary funding, we will need to delay, scale back, or eliminate some or all of our research and development programs and commercialization efforts; consider other various strategic alternatives, including a merger or sale; or cease operations. If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition, and results of operations could be materially adversely affected.

As a result of these factors, together with the anticipated continued spending that will be necessary to continue to research, develop, and commercialize the Company's product candidates, there is substantial doubt about the Company's ability to continue as a going concern within one year after the date that these consolidated financial statements are issued. The consolidated financial statements do not contain any adjustments that might result from the resolution of any of the above uncertainties.

2. Summary of Significant Accounting Policies

Basis of Presentation and Consolidation

The accompanying consolidated financial statements included herein have been prepared in conformity with GAAP and under the rules and regulations of the United States SEC. The consolidated financial statements include the accounts of Ocugen and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

In preparing the consolidated financial statements in conformity with GAAP, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the

date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to the inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include those used in the accounting for research and development contracts, including clinical trial accruals, determination of the collaborative arrangements transaction price, calculating the progress towards the satisfaction of the performance obligations under the collaborative arrangements, and determining the value of the non-cash consideration received under collaborative arrangements.

Segment Information

As of December 31, 2024, the Company viewed its operations and managed its business as one operating segment consistent with how the Company's chief operating decision-maker, the Company's Chief Executive Officer, makes decisions regarding resource allocation and assesses performance. As of December 31, 2024, substantially all of the Company's assets were located in the United States. Refer to Note 15 for additional information.

Cash, Cash Equivalents, and Restricted Cash

The Company considers all highly liquid investments that have maturities of three months or less when acquired to be cash equivalents. Cash equivalents may include bank demand deposits and money market funds that invest primarily in certificates of deposit, commercial paper, and United States government agency securities and treasuries. The Company records interest income received on its cash and cash equivalents to other income (expense), net in the consolidated statements of operations and comprehensive loss. The Company recorded $1.3 million and $2.5 million as interest income for the years ended December 31, 2024 and 2023, respectively. The Company's restricted cash balance as of December 31, 2024 consisted of cash held to collateralize a corporate credit card account and a line of credit related to an operating lease in the event of a payment default.

The following table provides a reconciliation of cash and restricted cash from the consolidated balance sheets to the total amount shown in the consolidated statements of cash flows (in thousands):

	As of December 31,	
	2024	2023
Cash	$ 58,514	$ 39,462
Restricted cash	307	—
Total cash and restricted cash	$ 58,821	$ 39,462

Fair Value Measurements

The Company follows the provisions of FASB ASC Topic 820, *Fair Value Measurements* ("ASC 820"), which 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 on the measurement date. ASC 820 also establishes a fair value hierarchy, which requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value. ASC 820 describes three levels of inputs that may be used to measure fair value:

Level 1 — quoted prices in active markets for identical assets or liabilities

Level 2 — quoted prices for similar assets and liabilities in active markets or inputs that are observable

Level 3 — inputs that are unobservable (for example, cash flow modeling inputs based on assumptions)

The carrying value of certain financial instruments, including cash, accounts payable, and accrued expenses, approximates their fair value due to the short-term nature of these instruments.

Marketable Securities

The Company accounts for marketable securities in accordance with FASB ASC Topic 320, *Investments — Debt and Equity Securities* ("ASC 320"). The Company determines the appropriate classification of its investments in debt securities at the time of purchase. Marketable securities with maturities of 90 days or less at the time of purchase are classified as cash equivalents on

the consolidated balance sheets. Debt securities are classified as trading securities if the security is bought and held primarily to be sold in the near term. Debt securities are classified as held-to-maturity if management has both the positive intent and ability to hold until the maturity of the security. Debt securities not classified as trading securities or held-to-maturity securities are classified as available-for-sale securities. The Company's marketable securities were previously comprised of debt securities and were classified as available-for-sale securities. The Company's marketable securities matured during the year ended December 31, 2023.

Available-for-sale securities are recorded at fair value based on inputs that are observable, either directly or indirectly, such as quoted prices for identical securities in active markets (Level 1) or quoted prices for similar securities in active markets or inputs that are observable (Level 2). Unrealized gains and losses are included in other comprehensive income (loss) in the consolidated statements of operations and comprehensive loss. Amortization of premium or accretion of discount on debt securities are included in other income (expense), net in the consolidated statements of operations and comprehensive loss.

The Company reviews investments in debt securities for other-than-temporary impairment if the fair value of the investment is less than the amortized cost basis. The assessment for other-than-temporary impairment is performed at the individual security level. After the assessment, if the Company does not have the intent and ability to hold the security until recovery of the unrealized loss, the difference between the fair value and amortized cost basis of the security is charged to results of operations resulting in a new amortized cost basis of the security. If the Company has the intent and ability to hold the security until recovery of the unrealized loss, the security is evaluated for potential credit losses. If a credit loss is deemed to exist, the credit loss is recognized in results of operations and an allowance for credit losses is recorded against the amortized cost basis of the security. In determining whether a credit loss exists related to an impaired debt securities, the Company considers, among other factors, the extent of the unrealized loss relative to the amortized cost basis, the credit rating of the issuer and any recent changes thereto, current and expected future economic conditions, and any adverse events or other changes in circumstances that have occurred which may indicate a potential credit loss. To date, the Company has not recognized any impairments with respect to its debt securities.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments — Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*. The FASB subsequently issued amendments to ASU No. 2016-13, which had the same effective date and transition date of January 1, 2023. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist of cash and restricted cash. The Company's cash and restricted cash are held in accounts at financial institutions that may exceed federally insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to significant credit risk beyond the standard credit risk associated with commercial banking relationships.

Property and Equipment, Net

The Company's property and equipment currently includes furniture and fixtures, machinery and equipment, leasehold improvements, and construction in progress. Property and equipment is recorded at historical cost less accumulated depreciation. Significant additions or improvements are capitalized, and expenditures for repairs and maintenance are charged to expense as incurred. Depreciation is calculated using the straight-line method and is recognized over the expected useful life of the underlying asset. Depreciation expense is included as research and development expense or general and administrative expense in the consolidated statements of operations and comprehensive loss based on the underlying nature of the associated asset. Construction in progress is not depreciated until such time that the asset is completed and placed into service.

Expected useful lives by major asset category are as follows:

Furniture and fixtures	3 to 7 years
Machinery and equipment	5 to 7 years
Leasehold improvements	Shorter of the expected useful life or remaining lease term

Leases

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company, if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company's lease agreements include lease and non-lease components, which the Company has elected not to account for separately for all classes of underlying assets. Lease expense for variable lease components is recognized when the obligation is probable.

The Company currently leases real estate classified as operating leases. Operating leases are included in other assets and operating lease obligations in the Company's consolidated balance sheets. At lease commencement, the Company records a lease liability based on the present value of the lease payments over the expected lease term including any options to extend the lease that the Company is reasonably certain to exercise and records a corresponding right-of-use lease asset based on the lease liability, adjusted for any lease incentives received and any initial direct costs paid to the lessor prior to the lease commencement date. Lease expense is recognized on a straight-line basis over the lease term and recognized as research and development expense or general and administrative expense based on the underlying nature of the expense. FASB ASC Topic 842, *Leases* ("ASC 842") requires a lessee to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. The implicit interest rates were not readily determinable in the Company's current operating leases. As such, the incremental borrowing rates were used based on the information available at the commencement dates in determining the present value of lease payments.

The lease term for the Company's leases includes the non-cancellable period of the lease plus any additional periods covered by either an option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Lease payments included in the measurement of the lease liability are comprised of fixed payments, variable payments that depend on an index or rate, and amounts probable to be payable under the exercise of an option to purchase the underlying asset if reasonably certain.

Variable payments not dependent on an index or rate and variable payments recognized if the index or rate changes that are associated with the Company's leases are recognized when the event, activity, or circumstance is probable. Variable payments include the Company's proportionate share of certain utilities and other operating expenses and are presented as operating expenses in the Company's consolidated statements of operations and comprehensive loss in the same line item as expense arising from fixed lease payments.

Impairment of Assets

The Company reviews its assets, including property and equipment, for impairment whenever changes in circumstances or events may indicate that the carrying amounts are not recoverable. These indicators include, but are not limited to, a significant change in the extent or manner in which an asset is used or its physical condition, a significant decrease in the market price of an asset, or a significant adverse change in the business or the industry that could affect the value of an asset. An asset is tested for impairment by comparing the net carrying value of the asset to the undiscounted net cash flows to be generated from the use and eventual disposition of the asset.

Stock-Based Compensation

The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718. The Company has issued stock-based compensation awards including stock options, RSUs, and PSUs, and also accounts for certain issuances of preferred stock and warrants in accordance with ASC 718. ASC 718 requires all stock-based payments, including grants of stock options, RSUs, and PSUs, to be recognized in the consolidated statements of operations and comprehensive loss based on their grant date fair values. The Company uses the Black-Scholes option-pricing model to determine the fair value of stock options granted. For RSUs, the fair value of the RSU is determined by the market price of a share of the Company's common stock on the grant date. For PSUs, the Company determines fair value by using a Monte Carlo simulation technique. The Company recognizes forfeitures as they occur.

Expense related to stock-based compensation awards granted with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Stock-based compensation awards generally vest over a one to three year requisite service period. Stock options have a

contractual term of 10 years. Expense for stock-based compensation awards with performance-based vesting conditions is only recognized when the performance-based vesting condition is deemed probable to occur. Expense for stock-based compensation awards with market-based and service-based vesting conditions is recognized ratably over the grantee's requisite service period. Compensation cost is not adjusted based on the actual achievement of the market-based performance goals. Expense related to stock-based compensation awards are recorded to research and development expense or general and administrative expense based on the underlying function of the individual that was granted the stock-based compensation award. Shares issued upon stock option exercise, PSU and RSU vesting are newly-issued common shares.

Estimating the fair value of stock options requires the input of subjective assumptions, including the expected term of the stock option, stock price volatility, the risk-free interest rate, and expected dividends. Estimating the fair value of PSUs requires the input of subjective assumptions, including stock price volatility, TSR ranking, the risk-free rate, and expected dividends. The assumptions used in the Company's Black-Scholes option-pricing model and Monte Carlo simulation technique represent management's best estimates and involve a number of variables, uncertainties, assumptions, and the application of management's judgment, as they are inherently subjective. If any assumptions change, the Company's stock-based compensation expense could be materially different in the future.

The assumptions used in Ocugen's Black-Scholes option-pricing model for stock options and in Ocugen's Monte Carlo simulation technique for PSUs are as follows, unless noted otherwise:

Expected Term. As Ocugen does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term, the expected term of employee stock options subject to service-based vesting conditions is determined using the "simplified" method, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the stock option. This expected term assumption is not an assumption used in the Company's Monte Carlo simulation technique for PSUs. The expected term of the PSUs is equal to the performance period of the PSUs.

Expected Volatility. The expected volatility is based on historical volatilities of Ocugen and similar entities within Ocugen's industry for periods commensurate with the assumed expected term, due to the lack of sufficient history of Ocugen.

Risk-Free Interest Rate. The risk-free interest rate is based on the interest rate payable on United States Treasury securities in effect at the time of grant for a period that is commensurate with the assumed expected term.

Expected Dividends. The expected dividend yield is 0% because Ocugen has not historically paid, and does not expect for the foreseeable future to pay, a dividend on its common stock.

TSR ranking. The Company's TSR, over a three-year period, is relative to the TSR, for that same period, as related to other companies within the Nasdaq Biotechnology index. This assumption is only used for the market-based PSUs.

Collaborative Arrangements and Revenue Recognition

The Company analyzes its collaborative arrangements to assess whether they are within the scope of ASC 808 to determine 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. This assessment is performed throughout the life of the arrangements based on changes to the arrangements. For collaborative arrangements within the scope of ASC 808 the Company may analogize to ASC 606 for certain elements.

The Company identifies the goods or services promised within each collaborative arrangement and assesses whether each promised good or service is distinct for the purpose of identifying the performance obligations in the contract. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that: (i) the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer and (ii) the entity's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

The allocation of the transaction price to the performance obligations in proportion to their standalone selling prices is determined at contract inception. If the consideration promised in a contract includes a variable amount, the Company estimates the amount of consideration to which it will be entitled in exchange for transferring the promised goods or services to a customer. The Company determines the amount of variable consideration by using the expected value method or the most likely amount method. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price is the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated

variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the period of adjustment.

In determining the transaction price, the Company adjusts consideration for the effects of the time value of money if the timing of payments provides the Company with a significant benefit of financing. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the counterparty and the transfer of the promised goods or services to the counterparty will be one year or less. The Company assessed its collaboration arrangements in order to determine whether a significant financing component exists and concluded that a significant financing component does not exist in any of its arrangements.

The Company recognizes as collaboration revenue the amount of the transaction price that is allocated to the respective performance obligation as each performance obligation is satisfied over time, with progress toward completion measured based on actual costs incurred relative to total estimated costs to be incurred over the life of the arrangement. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which the Company is expected to complete their performance obligations under the arrangements and in determining the estimated market value of the co-development services included in the transaction price. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. Adjustments to original estimates will be required as work progresses and additional information becomes known, even though the scope of the work required under the contract may not change. Any adjustment as a result of a change in estimates is made when facts develop, events become known, or an adjustment is otherwise warranted.

Under the Company's collaborative arrangements, the timing of revenue recognition and receipt of consideration may differ, and result in assets and liabilities. Assets represent revenues recognized in excess of the consideration received under collaborative arrangement. Liabilities represent the consideration received in excess of revenues recognized under collaborative arrangement.

Income Taxes

The Company uses the asset and liability method in accounting for income taxes. Deferred tax assets and liabilities are recorded for temporary differences between the tax basis of assets and liabilities and their reported amounts in the consolidated financial statements, using statutory tax rates in effect for the year in which the differences are expected to reverse. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the consolidated statements of operations and comprehensive loss in the period that includes the enactment date.

The Company evaluates its deferred tax assets each period to ensure that the estimated future taxable income will be sufficient in character, amount, and timing, to result in its realizability. A valuation allowance is recorded to reduce the carrying amounts of deferred tax assets, unless it is more likely than not that those assets will be realized. Management utilizes considerable judgment when establishing deferred tax valuation allowances. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences and carryforward deferred tax assets become deductible or utilized. The Company considers the scheduled reversal of taxable temporary differences, projected future taxable income, and tax planning strategies in making this assessment. As events and circumstances change, valuation allowances are adjusted within the consolidated statement of operations and comprehensive loss when applicable.

The Company recognizes net tax benefits under the recognition and measurement criteria of FASB ASC Topic 740, *Income Taxes*, which prescribes requirements and other guidance for financial statement recognition and measurement of positions taken or expected to be taken on tax returns. The Company recognizes a tax benefit for positions taken for tax return purposes when it will be more likely than not that the positions will be sustained upon tax examination, based solely on the technical merits of the tax positions. Otherwise, no tax benefit is recognized. The tax benefits recognized are measured based on the largest benefit that has a greater than 50% likelihood of being realized upon ultimate settlement. The Company recognizes potential accrued interest and penalties related to unrecognized tax benefits as a component of income tax expense in the consolidated statement of operations and comprehensive loss. Tax examinations are often complex, as tax authorities may disagree with the treatment of items reported by the Company and may require several years to resolve. As a result, the Company's provision for income taxes is recorded on the basis of available information, but amounts recorded may be impacted as a result of future examinations.

Recently Adopted Accounting Standards

In August 2020, the FASB issued ASU No. 2020-06, *Debt — Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging — Contracts in Entity's Own Equity (Subtopic 815-40)*. This standard will have an effective and transition date of January 1, 2024. This standard simplifies an issuer's accounting for convertible instruments by eliminating

two of the three models that require separate accounting for embedded conversion features as well as simplifies the settlement assessment that entities are required to perform to determine whether a contract qualifies for equity classification. This standard also requires entities to use the if-converted method for all convertible instruments in the diluted earnings per share calculation and includes the effect of potential share settlement (if the effect is more dilutive) for instruments that may be settled in cash or shares, except for certain liability-classified share-based payment awards. The standard requires new disclosures about events that occur during the reporting period that cause conversion contingencies to be met and about the fair value of a public business entity's convertible debt at the instrument level, among other things. The adoption of ASU 2020-06 on January 1, 2024 did not have a material impact on the Company's consolidated financial statements and related disclosures.

In November 2023, the FASB issued ASU 2023-07 “Segment Reporting: Improvements to Reportable Segment Disclosures”. This guidance expands public entities’ segment disclosures primarily by requiring disclosure of significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss, an amount and description of its composition for other segment items, and interim disclosures of a reportable segment’s profit or loss and assets. The guidance is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The amendments are required to be applied retrospectively to all prior periods presented in an entity’s financial statements. The Company adopted the guidance in the fiscal year beginning January 1, 2024. There was no impact on the Company's reportable segments identified and additional required disclosures have been included in Note 15.

Recent Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09 “Income Taxes (Topic 740): Improvements to Income Tax Disclosures”. This guidance is intended to enhance the transparency and decision-usefulness of income tax disclosures. The amendments in ASU 2023-09 address investor requests for enhanced income tax information primarily through changes to disclosure regarding rate reconciliation and income taxes paid both in the United States and in foreign jurisdictions. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024 on a prospective basis, with the option to apply the standard retrospectively. Early adoption is permitted. The Company is currently evaluating this guidance to determine the impact it may have on its consolidated financial statements disclosures.

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 (ASU 2024-03). The new guidance requires disaggregated information about certain income statement expense line items on an annual and interim basis. This guidance will be effective for annual periods beginning the year ended December 31, 2027 and for interim periods thereafter. The new standard permits early adoption and can be applied prospectively or retrospectively. We are evaluating the effect that this guidance will have on our consolidated financial statements and related disclosures.

ASU 2024-04, Debt with Conversion and Other Options (Subtopic 470-20): Induced Conversions of Convertible Debt Instruments. In November 2024, the FASB issued ASU 2024-04, which clarifies the requirements for determining whether certain settlements of convertible debt instruments should be accounted for as induced conversions rather than as debt extinguishments. This update is effective for annual periods beginning after December 15, 2025, including interim periods within those fiscal years, though early adoption is permitted. We are evaluating the effect that this guidance will have on our consolidated financial statements and related disclosures.

3. License and Development Agreements

Co-Development and Commercialization Agreement with CanSino Biologics, Inc.

The Company entered into a co-development and commercialization agreement with our collaboration partner CanSino Biologics, Inc. ("CanSinoBIO") with respect to the development and commercialization of the Company's modifier gene therapy product candidates, OCU400, OCU410, and OCU410ST. The co-development and commercialization agreement was originally entered into in September 2019 ("the Original CanSinoBIO Agreement") with regards to OCU400 and was subsequently amended in September 2021 and November 2022 ("the Amendments"), to include OCU410 and OCU410ST, respectively. The Company concluded that the Original CanSinoBIO Agreement and the Amendments are separate agreements (collectively referred to as the "CanSinoBio Agreements"). Pursuant to the CanSinoBIO Agreements, the Company and CanSinoBIO are collaborating on the development of the Company's modifier gene therapy platform. CanSinoBIO is responsible for the chemistry, manufacturing, and controls development and manufacture of clinical supplies of such products and is responsible for the costs associated with such activities. CanSinoBIO has an exclusive license to develop, manufacture, and commercialize the Company's modifier gene therapy platform in and for the CanSinoBIO Territory, and the Company

maintains exclusive development, manufacturing, and commercialization rights with respect to the Company's modifier gene therapy platform outside the Company Territory.

Should any of the product candidates be commercialized in the CanSinoBIO Territory, CanSinoBIO will pay to the Company an annual royalty between mid- and high-single digits based on Net Sales (as defined in the CanSinoBIO Agreements) of the products included in the Company's modifier gene therapy platform in the CanSinoBIO Territory. The Company will pay to CanSinoBIO an annual royalty between low- and mid-single digits based on Net Sales of the products included in the Company's modifier gene therapy platform in the Company Territory.

Accounting analysis and revenue recognition

The Company determined the collaboration arrangements with CanSinoBIO, are within the scope of ASC 808 and has analogized to ASC 606 to account for CanSinoBIO's access to its IP as well as data generated in connection with the co-development activities to be undertaken by Ocugen. These elements of the arrangements are not distinct and are accounted for as a single performance obligation.

The non-cash consideration to be received related to the Company's satisfaction of the performance obligation includes but is not limited to services related to chemistry, manufacturing, and controls development and manufacture of clinical supplies of such products through completion of pre-clinical, clinical, regulatory, and other commercialization readiness services. The estimated market value of the co-development services to be performed by CanSinoBIO, represents variable consideration that is included in the transaction price. The Company recognizes collaborative arrangement revenue over time using an input method using ratio of costs incurred to date compared to total estimated costs required to satisfy the performance obligation under the CanSinoBIO Agreements.

The Company constrained the transaction price related to certain future co-development services and future royalties, as it assessed that it is probable that the inclusion of such variable consideration could result in a significant reversal of cumulative revenue in future periods. The variable consideration, which is based on continued successful development of our programs, is reevaluated at each reporting period and as changes in circumstances occur.

The services provided by CanSinoBIO are recorded as research and development expense as incurred and the difference between the revenue and expense recognized is recorded on the Company's balance sheet as a contract liability within Accrued expenses and other current liabilities. The related revenue recognized was recorded in the consolidated statements of operations and comprehensive loss as collaborative arrangement revenue and was approximately $4.1 million and $6.0 million for the year ended December 31, 2024 and the year ended December 31, 2023, respectively. The related expense incurred for services provided by CanSinoBIO was recorded in the consolidated statements of operations and comprehensive loss as research and development expense and was approximately $1.9 million and $5.3 million for the year ended December 31, 2024 and the year ended December 31, 2023, respectively.

The contract liability was $8.4 million and $10.5 million as of December 31, 2024 and December 31, 2023, respectively. Revenue recognized for the year ended December 31, 2024, that was included in the contract liabilities balances as of January 1, 2024 was approximately $4.1 million. Revenue recognized for the year ended December 31, 2023, that was included in the contract liabilities balances as of January 1, 2023, was approximately $6.0 million.

NIAID Project NextGen Clinical trial support

In October 2023, OCU500 was selected by the National Institute of Allergy and Infectious Diseases ("NIAID") Project NextGen for inclusion in clinical trials. Project NextGen is a $5 billion multi-government agency initiative to develop the next generation of vaccines and therapeutics to combat the spread of COVID-19. NIAID, with funding from Project NextGen, will cover the full cost of the clinical trials, including operations and related analysis. Ocugen will be responsible for providing clinical trial materials and upon completion will have full right of reference to the findings, which Ocugen believes will provide clinical evidence to support the further development of the Company’s lead mucosal vaccine candidate. NIAID intends to initiate a Phase 1 clinical trial in the second quarter of 2025.

Exclusive License Agreement with Washington University

In September 2022, the Company entered into the WU License Agreement with Washington University, pursuant to which the Company was granted an exclusive, sublicensable, royalty-bearing license to patent rights for a mucosal COVID-19 vaccine, as well as a license to certain tangible research property and technical information necessary to exploit the patent rights within the United States, Europe, and Japan. The Company paid Washington University an initial license issuance fee of $1.0 million,

which was recognized as research and development expense in the consolidated statement of operations and comprehensive loss during the year ended December 31, 2022. In January 2023, the Company amended the WU License Agreement to add the countries of South Korea, Australia, and China to the Mucosal Vaccine Territory, and in November 2023, the Company further amended the WU License Agreement to add Hong Kong to the Mucosal Vaccine Territory. The Company is required to pay Washington University an annual license maintenance fee, payments upon the achievement of certain development and commercial milestones in the aggregate amount of up to $37.0 million, and low single-digit percentage royalties on Net Sales of licensed products (as defined in the WU License Agreement).

Pursuant to the WU License Agreement, the Company may make, have made, sell, offer for sale, use, market, promote, distribute, export, and import licensed products in the Mucosal Vaccine Territory. The Company will use commercially reasonable efforts to develop, manufacture, promote, and sell the licensed products in the Mucosal Vaccine Territory.

Washington University maintains control of patent preparation, filing, prosecution, and maintenance. The Company is responsible for Washington University's out-of-pocket expenses related to the preparation, filing, prosecution, issuance, and maintenance of the licensed patent rights incurred pursuant to the WU License Agreement.

The WU License Agreement will expire on a country-by-country basis and a licensed product-by-licensed product basis and end, separately in each such country and for each such licensed product, upon the latter of (a) the expiration date of the last valid claim, (b) the fifteenth (15th) anniversary of the date of the first commercial sale of a licensed product, or (c) the expiration of the last form of market exclusivity (as defined in the WU License Agreement), subject to the earlier termination of the WU License Agreement in accordance with its terms. In addition, the Company may terminate the WU License Agreement without cause by giving at least 90 days written notice. The WU License Agreement contains customary termination provisions in the event of an uncured material breach or upon certain corporate actions, including bankruptcy, receivership, or liquidation.

Exclusive License Agreement with The Schepens Eye Research Institute, Inc.

In December 2017, the Company entered into an exclusive license agreement with The Schepens Eye Research Institute, Inc. ("SERI"), which was amended in January 2021 (as so amended, the "SERI Agreement"). The SERI Agreement gives the Company an exclusive, worldwide, sublicensable license to patent rights, biological materials, and technical information for NHR genes Nuclear Receptor Subfamily 1 Group D Member 1 (*"NR1D1"*), NR2E3 (OCU400), *RORA* (OCU410 and OCU410ST), Nuclear Protein 1, Transcriptional Regulator (*"NUPR1"*), and Nuclear Receptor Subfamily 2 Group C Member 1 (*"NR2C1"*). The January 2021 amendment to the SERI Agreement additionally granted the Company rights in co-owned intellectual property pursuant to certain patents and provisional patents at the time of the amendment. Under the SERI Agreement, the Company may make, have made, use, offer to sell, sell, and import licensed products, and must use commercially reasonable efforts to bring one or more licensed products to market as soon as reasonably practicable.

SERI maintains control of patent preparation, filing, prosecution, and maintenance. The Company is responsible for SERI's out-of-pocket expenses related to the filing, prosecution, and maintenance of the licensed patent rights. In the event that SERI decides to discontinue the prosecution or maintenance of the licensed patent rights, the Company has the right, but not the obligation, to file for, or continue to prosecute, maintain, or enforce such licensed patent rights. The Company has assumed prosecution of certain licensed patent rights under the SERI Agreement. The Company has paid $75 thousand related to annual maintenance and $750 thousand in milestone payments for the year ended December 31, 2024.

Exclusive License Agreement with the University of Colorado

In March 2014, the Company entered into an exclusive license agreement with the University of Colorado ("CU"), which was amended in January 2017 and clarified by a letter of understanding in November 2017 (as so amended and clarified, the "CU Agreement"). The CU Agreement gives the Company an exclusive, worldwide, sublicensable license to patents for OCU200 to make, have made, use, import, offer to sell, sell, have sold, and practice the licensed products in all therapeutic applications. Under the CU Agreement, the Company must use commercially reasonable efforts to develop, manufacture, sublicense, market, and sell the licensed products, and has assumed primary responsibility for preparing, filing, and prosecuting broad patent claims for OCU200 for CU's benefit. Further, the Company assumed primary responsibility for all patent activities, including all costs associated with the perfection and maintenance of the patents for OCU200. The Company has paid $20 thousand related to annual maintenance for the year ended December 31, 2024.

License Agreement with Purpose Co. Ltd.

In December 2005, Histogenics Corporation ("Histogenics") entered into an exclusive agreement (the "Purpose Agreement") to sublicense certain technology from Purpose Co. Ltd. ("Purpose"), which the Company assumed as a result of its reverse merger with Histogenics. Purpose entered into the original license agreement ("BWH-Purpose Agreement") with Brigham and

Women's Hospital, Inc. ("BWH") in August 2001. The BWH-Purpose Agreement granted Purpose an exclusive, royalty-bearing, worldwide, sublicensable license, under its rights in licensed patents and patent applications co-owned by BWH and Purpose to make, use, and sell (1) an apparatus for cultivating a cell or tissue, (2) cell or tissue products made using such apparatus, (3) cell or tissue products made using processes for cultivating a cell or tissue as disclosed in the licensed patents and patent applications, and (4) any apparatus that cultivates cells or tissues using such processes, in each case, whose manufacture, use, or sale is covered by a valid claim of the licensed patents and patent applications, only for therapeutic use.

The Purpose Agreement was amended and restated in June 2012, pursuant to which Purpose granted Histogenics outside of Japan: (i) exclusive rights to all of Purpose's technology (owned or licensed) related to the exogenous tissue processors, which is used in the development of NeoCart, (ii) continued supply of exogenous tissue processors, and (iii) rights to manufacture the exogenous tissue processors at any location the Company chooses. In exchange for such consideration, Purpose was granted an exclusive license in Japan for the use of all of the Company's NeoCart technology and was reimbursed for development costs on a multi-unit exogenous tissue processor. In May 2016, the Purpose Agreement was amended, whereby Histogenics reacquired the development and commercialization rights to NeoCart in Japan.

The Purpose Agreement, as amended, provides the Company with the ability, worldwide, to (i) use, make, have made, sell, offer for sale, import or otherwise exploit products or services covered by claims of Purpose's patents and (ii) use, reproduce, modify, create derivative works of and otherwise exploit Purpose's technology for the design, development, manufacture, testing, support, and commercialization of any product or service that incorporates or builds upon Purpose's technology, in each case, only in connection with articular cartilage, ligaments, tendons, and meniscus. Purpose retains the right to sell its single unit exogenous tissue processer machines to research institutes for general but noncommercial use anywhere in the world.

4. Fair Value Measurements

The Company believes the fair value using Level 2 inputs of the borrowings under the EB-5 Loan Agreement and Avenue Capital Loan and Security Agreement (as defined in Note 8) approximate their carrying value. See Note 8 for additional information.

5. Property and Equipment

During the year ended December 31, 2024, leasehold improvements associated with the Company's GMP facility, which had been previously classified as construction in progress, were placed into service. The following table provides a summary of the major components of property and equipment as reflected on the consolidated balance sheets (in thousands):

	As of December 31,	
	2024	2023
Furniture and fixtures	$ 433	$ 337
Machinery and equipment	3,192	1,557
Leasehold improvements	16,089	2,086
Construction in progress	—	14,540
Total property and equipment	19,714	18,520
Less: accumulated depreciation	(3,160)	(1,230)
Total property and equipment, net	$ 16,554	$ 17,290

Depreciation expense was $1.9 million and $0.7 million during the years ended December 31, 2024 and 2023, respectively.

6. Operating Leases

The Company has commitments under operating leases for office, laboratory, and manufacturing space in Malvern, Pennsylvania and other locations. The Company's corporate headquarters, located in Malvern, Pennsylvania, lease has an initial term of approximately seven years and includes options to extend the lease for up to 10 years, which the Company has not elected to account for since it is not reasonably certain that the Company will exercise such option. The Company's current GMP facility, located in Malvern, Pennsylvania, lease has an initial term of seven years and includes an option to extend the lease for up to five years, which the Company has elected to account for since it is reasonably certain that the Company will exercise such option. The Company leases two other general use facilities, within the United States, which have initial terms of two to three years and contain no option to extend.

The components of lease expense were as follows (in thousands):

	Year ended December 31,	
	2024	2023
Operating lease cost	$ 893	$ 784
Variable lease cost	355	342
Total lease cost	$ 1,248	$ 1,126

Supplemental balance sheet information related to leases was as follows (in thousands):

	As of December 31,	
	2024	2023
Right-of-use assets, net	$ 3,613	$ 3,944
Current lease obligations	$ 519	$ 574
Non-current lease obligations	3,313	3,567
Total lease liabilities	$ 3,832	$ 4,141

Supplemental information related to leases was as follows:

	Year ended December 31,	
	2024	2023
Weighted-average remaining lease terms (years)	5.9	7.0
Weighted-average discount rate	9.4 %	9.3 %

Future minimum base rent payments are approximately as follows (in thousands):

For the years ending December 31,	Amount
2025	$ 877
2026	894
2027	867
2028	884
2029	705
Thereafter	978
Total	$ 5,206
Less: present value adjustment	(1,374)
Present value of minimum lease payments	$ 3,832

7. Accrued Expenses and Other Current Liabilities

The following table provides a summary of the major components of accrued expenses and other current liabilities as reflected on the consolidated balance sheets (in thousands):

	As of December 31,	
	2024	2023
Research and development	$ 160	$ 212
Clinical	740	84
Professional fees	977	580
Employee-related	2,433	1,791
Deferred revenue relating to collaborative arrangements	8,368	10,525
Other	2,822	151
Total accrued expenses and other current liabilities	$ 15,500	$ 13,343

8. Debt

In September 2016, in connection with the United States government's foreign national investor program, commonly known as the EB-5 Program, the Company entered into the EB-5 Loan Agreement which provided for cumulative borrowings of up to $10.0 million from EB-5 Life Sciences as the lender. Pursuant to the EB-5 Loan Agreement, borrowings were made in $0.5 million increments with a fixed interest rate of 4.0% per annum (the "Original Offering"). The borrowings pursuant to the Original Offering are secured by substantially all of the Company's assets, with the exception of any patents, patent applications, pending patents, patent licenses, patent sublicenses, trademarks, and other intellectual property rights held by the Company.

Under the terms and conditions of the Original Offering, the Company borrowed $1.0 million during 2016, $0.5 million during 2020, $0.5 million in September 2022, and an additional $0.5 million in May 2023. Issuance costs were recognized as a reduction to the loan balance and are amortized to interest expense over the term of each borrowing. Pursuant to the Original Offering, each outstanding borrowing, including accrued interest, becomes due upon the seventh anniversary of its disbursement date, subject to certain extension provisions. In January 2024, the Company entered into an agreement to extended the current portion of borrowings owed under the EB-5 Loan Agreement to March 2025. Once repaid, amounts cannot be re-drawn.

The March 2022 EB-5 Reform and Integrity Act of 2022 (the "RIA") enacted changes to the EB-5 Program, including but not limited to: raising the minimum investment amount for a targeted employment area (the "TEA") from its previous level of $0.5 million to its new level of $0.8 million, as well as modifying the process for the creation of TEAs. Under the previous regime, the state in which the TEA would be located could send a letter in support of efforts to designate a TEA. Under the current regime, only United States Citizenship and Immigration Services can designate TEAs.

In connection with the aforementioned changes to the EB-5 Program, the Original Offering was amended in May 2023 (the "Amended Offering"). Pursuant to the terms and conditions of the Amended Offering, EB-5 Life Sciences now provides for cumulative borrowings of up to $20.0 million. Future borrowings can be made in increments of $0.8 million with a fixed interest rate of 4.0% per annum. Each future borrowing pursuant to the Amended Offering, including accrued interest, will become due upon the seventh anniversary of its disbursement date. The Company has not made any borrowings pursuant to the Amended Offering as of December 31, 2024.

The carrying values of the borrowings pursuant to the Original Offering as of December 31, 2024 and 2023 are summarized below (in thousands):

	As of December 31,	
	2024	2023
Principal outstanding	$ 2,500	$ 2,500
Plus: accrued interest	500	400
Less: unamortized debt issuance costs	(84)	(100)
Carrying value, net	2,916	2,800
Less: current portion of long term debt	(1,326)	—
Long term debt, net of current portion	$ 1,590	$ 2,800

In November 2024, the Company entered into a debt financing transaction with Avenue Capital for net proceeds of $29.2 million. The loan has a maturity date of November 1, 2028, of which the first 24 months are interest only, and bears interest at a variable rate per annum equal to the greater of the Prime Rate plus 4.25% or 12.25%. Additionally, the Lender has the right to convert an aggregate amount of up to $6.0 million of the outstanding principal amount into shares of Common Stock at a conversion price per share equal to a 80% of the trading price on the date of conversion, which shall be at Lenders' option. In the event the Company elects to prepay the Term Loans in full, Lenders shall have 10 days to elect to exercise its conversion right prior to such prepayment. All conversion rights shall terminate on Term Loans payoff. Notwithstanding the foregoing, the aggregate amount of Common Stock issued pursuant to the “Conversion Right” and the “Equity Grant” shall not exceed a number of shares equal to 19.9% of the Company’s outstanding Common Stock. The agreement is collateralized by all of the Company’s assets in which the Agent is granted senior secured lien. The Company also granted the Lenders a negative pledge on the Company’s intellectual property. In connection with the debt financing transaction, the Company entered into a Subscription Agreement with Avenue Capital, pursuant to which the Company issued 1,056,338 shares of Common Stock to Avenue Capital with an issue date of November 6, 2024.

The carrying values of the borrowings pursuant to the Loan and Security Agreement as of December 31, 2024 and 2023 are summarized below (in thousands):

	As of December 31,	
	2024	2023
Principal outstanding	$ 30,000	$ —
Plus: accrued interest	—	—
Less: unamortized debt issuance costs	(4,245)	—
Carrying value, net	$ 25,755	$ —

The following table summarizes the scheduled debt maturities for the next five years and thereafter (in thousands):

For the years ending December 31,	Total Maturities
2025	$ 1,000
2026	1,250
2027	16,000
2028	13,750
2029 and thereafter	500
Total Debt	$ 32,500

9. Equity

Offerings of Common Stock

Public Offering

In July 2024, the Company entered into an underwriting agreement with an underwriter, pursuant to which the Company agreed to issue and sell to the underwriter in a public offering (the "July 2024 Public Offering") 30.4 million shares of its common stock, par value $0.01 per share, at a public offering price of $1.15 per share (the "Offering Price"). Pursuant to the terms of the underwriting agreement, the Company granted to the underwriter a 30-day option to purchase up to an additional 4,565,217 shares of common stock at the offering price (the "Option Shares") at the public offering price, less underwriting discounts and commissions. The offering closed in August 2024. The net proceeds to the Company from the offering, excluding any exercise by the underwriter of its 30-day option to purchase any of the option shares, were $32.3 million after deducting the underwriting discounts and commissions and offering expenses paid to the Company. The July 2024 Public Offering was made pursuant to the Company's Registration Statement on Form S-3 and a prospectus supplement, which was previously filed with the SEC and became effective on May 1, 2024. In August 2024, the underwriter exercised their option to purchase 2,282,608 Option Shares at the Offering Price. The net proceeds to the Company from the exercise of the underwriter's option were $2.4 million after deducting the underwriting discounts and commissions and offering expenses paid to the Company.

Increase in Capital Stock

In July 2024, the Company's certificate of incorporation was amended to increase the total number of shares of all classes of stock the Company has authority to issue to four hundred million shares. This consists of three hundred ninety million shares of Common Stock, par value $0.01 per share (the "Common Stock"), and ten million shares of Preferred Stock, par value $0.01 per share ("the Preferred Stock").

COVAXIN Preferred Stock Purchase Agreement

On March 1, 2021, the Company entered into a stock purchase agreement (the "Stock Purchase Agreement") with Bharat Biotech International Limited ("Bharat Biotech"), pursuant to which the Company agreed to issue and sell 0.1 million shares of the Company's Series B Convertible Preferred Stock, par value $0.01 per share (the "Series B Convertible Preferred Stock"), at a price per share equal to $109.60, to Bharat Biotech. On March 18, 2021, the Company issued the Series B Convertible Preferred Stock as an advance payment of $6.0 million for the supply of COVAXIN, a monovalent vaccine, to be provided by Bharat Biotech pursuant to a Development and Commercial Supply Agreement (the "Supply Agreement").

Each share of Series B Convertible Preferred Stock was convertible, at the option of Bharat Biotech, into 10 shares of the Company's common stock (the "Conversion Ratio") only after (i) the Company received stockholder approval to increase the number of authorized shares of common stock under its Sixth Amended and Restated Certificate of Incorporation, which the Company received in April 2021, and (ii) the Company's receipt of shipments by Bharat Biotech of the first 10.0 million doses of COVAXIN manufactured by Bharat Biotech pursuant to the Supply Agreement, and further on the terms and subject to the conditions set forth in the Certificate of Designation of Preferences, Rights and Limitations of Series B Convertible Preferred Stock. The conversion rate of the Series B Convertible Preferred Stock was subject to adjustment in the event of a stock dividend, stock split, reclassification, or similar event with respect to the Company's common stock. In May 2024, Bharat Biotech and the Company entered into a Stock Forfeiture Agreement whereby the outstanding shares of Series B Convertible Preferred Stock were redeemed.

The Company accounted for the issuance of the Series B Convertible Preferred Stock in accordance with ASC 718 and recorded its grant date fair value of $5.0 million within stockholders' equity during the year ended December 31, 2021, with a corresponding short-term asset for the advanced payment for the supply of COVAXIN included in prepaid expenses and other current assets in the consolidated balance sheet as of December 31, 2021. The Company utilized the traded common stock price, adjusted by the Conversion Ratio, to value the Series B Convertible Preferred Stock and the Finnerty model to estimate a 15% discount rate for the lack of marketability of the instrument. The valuation incorporated Level 3 inputs in the fair value hierarchy, including the estimated time until the instrument's liquidity and estimated volatility of the Company's common stock as of the grant date. As of December 31, 2022, the remaining balance of the short-term asset for the advanced payment for the supply of COVAXIN was $4.1 million.

In April 2023, the FDA announced the cancellation of all emergency use authorizations ("EUA") issued with respect to monovalent COVID-19 vaccine formulations. Consequently, the Company determined it was no longer commercially viable to further the development of COVAXIN in its North American territories. During the year ended December 31, 2023, the

Company wrote off the remaining balance of the short-term asset for the advanced payment for the supply of COVAXIN of $4.1 million to research and development expense in the consolidated statements of operations and comprehensive loss. As referenced above, in May 2024, the outstanding shares of Series B Convertible Preferred Stock were reacquired.

10. Warrants

Beginning in 2016, the Company issued warrants to purchase common stock. As of December 31, 2024 and 2023, 0.6 million warrants were vested and outstanding. As of December 31, 2024, the outstanding warrants had a weighted-average exercise price of $6.23 per share and expire between 2026 and 2027.

11. Stock-Based Compensation

Stock-based compensation expense for stock options, RSUs, and PSUs is reflected in the consolidated statements of operations and comprehensive loss as follows (in thousands):

	Year ended December 31,	
	2024	2023
General and administrative	$ 5,269	$ 6,876
Research and development	2,158	2,341
Total	$ 7,427	$ 9,217

As of December 31, 2024, the Company had $5.4 million of unrecognized stock-based compensation expense related to stock options, RSUs and PSUs outstanding, which is expected to be recognized over a weighted average period of 1.6 years.

Equity Plans

The Company maintains two equity compensation plans, the 2014 Ocugen OpCo, Inc. Stock Option Plan (the "2014 Plan") and the Ocugen, Inc. 2019 Equity Incentive Plan (the "2019 Plan", collectively with the 2014 Plan, the "Plans"). On the first business day of each fiscal year, pursuant to the "Evergreen" provision of the 2019 Plan, the aggregate number of shares that may be issued under the 2019 Plan will automatically increase by a number equal to the lesser of 4% of the total number of shares of the Company's common stock outstanding on December 31st of the prior year, or a number of shares determined by the Board. As of December 31, 2024, the 2014 Plan and the 2019 Plan authorize for the granting of up to 0.8 million and 38.6 million equity awards in respect to the Company's common stock, respectively. The 2014 Plan and 2019 Plan have 0.5 million and 16.1 million equity awards remaining available for future grant, respectively, as of December 31, 2024. In addition to stock options, PSUs and RSUs granted under the Plans, the Company has granted certain stock options and RSUs as material inducements to employment in accordance with Nasdaq Listing Rule 5635 (c)(4), which were granted outside of the Plans.

Stock Options to Purchase Common Stock

The assumptions utilized in the fair value calculations for stock options as of December 31, 2024 and 2023 were as follows:

	Year ended December 31,	
	2024	2023
Weighted average expected option term (years)	5.9	5.9
Range of expected stock price volatility	104% – 112%	103% – 109%
Weighted average expected stock price volatility	108%	106%
Range of risk-free interest rate	3.4% – 4.6%	3.5% – 4.4%
Expected dividend rate	0%	0%

The following table summarizes the stock option activity:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (In Thousands)
Options outstanding at December 31, 2023	13,161,228	$ 2.38	7.86	$ 337
Granted	4,055,895	0.96		
Exercised	(284,788)	0.57		183
Forfeited	(735,187)	3.52		
Options outstanding at December 31, 2024	16,197,148	$ 2.01	7.40	$ 1,128
Vested and expected to vest at December 31, 2024	16,197,148	$ 2.01	7.40	$ 1,128
Options exercisable at December 31, 2024	9,687,991	$ 2.40	6.60	$ 731

The weighted average grant date fair values of stock options granted during the years ended December 31, 2024 and 2023 were $0.80 and $0.85, respectively. During the years ended December 31, 2024 and 2023, the Company received $0.2 million and $0.1 million of cash proceeds from the exercises of stock options, respectively.

RSUs

The following table summarizes the RSU activity:

	Number of Shares	Weighted Average Grant-Date Fair Value	Aggregate Intrinsic Value (In Thousands)
RSUs unvested at December 31, 2023	2,982,661	$ 1.63	$ 1,715
Granted	39,738	0.51	22
Vested	(1,063,537)	1.85	768
Forfeited	(56,405)	1.64	59
RSUs unvested at December 31, 2024	1,902,457	$ 1.49	$ 1,531

PSUs

In December 2023, pursuant to the 2019 Plan, the Compensation Committee of the Board adopted a performance restricted stock unit agreement (the "PSU Agreement"). Pursuant to the PSU Agreement, the Company granted 615,467 and 256,885 of market-based performance stock units at target on January 2, 2024 and April 16, 2024, respectively. All of these PSUs cliff vest after the requisite service period ending on December 31, 2026. The PSUs have the potential to be earned at between 0% and 200% of the number of awards granted depending on the level of growth of the Company's TSR as compared to the TSR of the

companies within the Nasdaq Biotechnology Index over the performance period. The fair value of the market-based PSUs was determined using a Monte Carlo simulation technique.

The following table summarizes the unvested PSU activity:

	Number of Shares	Weighted Average Grant-Date Fair Value
PSUs unvested at December 31, 2023	—	$ —
Granted	872,352	$ 1.71
Vested	—	$ —
Forfeited	—	$ —
PSUs unvested at December 31, 2024	872,352	$ 1.71

12. Income Taxes

The Company's losses before income taxes and provision (benefit) for income taxes is as follows (in thousands):

	Year ended December 31,	
	2024	2023
Loss before income taxes	$ (54,054)	$ (63,078)
Provision (benefit) for income taxes	—	—

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

	Year ended December 31,	
	2024	2023
Expected provision at statutory rate	21.0 %	21.0 %
State income tax, net of federal benefit	5.9 %	6.4 %
Tax credits	3.7 %	3.5 %
Change in state tax rate	(2.4)%	(2.8)%
Expired Capita Loss	(13.2)%	— %
Other, net	(2.0)%	(1.6)%
Change in valuation allowance	(13.0)%	(26.5)%
Effective tax rate	— %	— %

The Company's deferred tax assets (liabilities) are comprised of the following (in thousands):

	As of December 31,	
	2024	2023
Deferred tax assets:		
Net operating loss carryforwards	$ 67,818	$ 58,324
Capital loss carryforwards	—	7,298
Start-up costs	9,699	9,699
Accruals and reserves	672	276
Intellectual property	4,074	4,565
Stock-based compensation	3,063	2,873
Capitalization of research and development expense	20,800	18,702
Deferred revenue	1,784	1,293
Tax credits	11,641	9,324
Lease liabilities	966	977
Total deferred tax assets	120,517	113,331
Valuation allowance	(119,616)	(112,413)
Deferred tax assets, net of valuation allowance	$ 901	$ 918
Deferred tax liabilities:		
Lease right-of-use assets	(901)	(918)
Net deferred tax assets	$ —	$ —

The Company's valuation allowance increased during 2024 by approximately $7.2 million primarily due to Section 174 expenditure capitalization. The Company has evaluated both positive and negative evidence when assessing the realizability of its deferred tax assets. Management has considered the Company's history of cumulative net losses, estimated future taxable income as well as tax planning strategies and has concluded that it is more likely than not that the Company will not realize the benefits of its deferred tax assets. Accordingly, a full valuation allowance has been established against these net deferred tax assets as of December 31, 2024 and 2023, respectively.

As of December 31, 2024 and 2023, the Company had United States federal net operating loss ("NOL") carryforwards of $263.9 million and $226.2 million, respectively, which may be available to offset future income tax liabilities. The 2017 Tax Cut and Jobs Act generally allows federal losses generated after 2017 to be carried forward indefinitely, but also limits the NOL deduction to the lesser of the NOL carryover or 80% of a corporation's taxable income (subject to Section 382 of the Internal Revenue Code of 1986, as amended ("IRC")). Additionally, there is no carryback for losses generated after 2017. Losses generated prior to 2018 are deductible using the lesser of a corporation's NOL carryover or 100% of a corporation's taxable income and have a 20 year carryforward period. The Company has federal NOLs generated after 2017 of $211.2 million, which do not expire. The federal NOLs generated prior to 2018 of $52.7 million will expire at various dates through 2037. In addition, the Company had all capital loss carryforwards expire in 2024.

As of December 31, 2024 and 2023, the Company had United States state NOL carryforwards of $262.2 million and $224.5 million, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2043. As of December 31, 2024 and 2023, the Company had federal tax credit carryforwards of approximately $11.5 million and $9.2 million, respectively, which are available to offset future federal tax liabilities which expire at various dates through 2043. As of December 31, 2024 and 2023, the Company had state tax credit carryforwards of approximately $0.2 million and $0.1 million, respectively, which are available to reduce future tax liabilities and expire at various dates through 2034.

NOL and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and relevant state tax authorities. Utilization of NOL and tax credit carryforwards may be subject to a substantial annual limitation under Section 382 and Section 383 of the IRC and corresponding provisions of state law, due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of NOL and tax credit carryforwards that can be utilized annually to offset future federal and state tax liabilities.

The Company has not yet conducted a comprehensive study to assess whether any ownership change has occurred since its inception. A limitation may result in the expiration of a portion of the NOL or tax credit carryforwards before utilization, which would be offset by a change in the Company's valuation allowance. Until a study is completed by the Company, no NOL carryforward amounts will be offset by an unrecognized tax benefit related to Section 382.

A full valuation allowance has also been recorded against the Company's tax credits. If an adjustment is required, it would be offset by a corresponding change in the valuation allowance.

A reconciliation of the beginning and ending amount of unrecognized tax benefits is as follows (in thousands):

	Year ended December 31,	
	2024	2023
Gross unrecognized tax benefits at beginning of year	$ 303	$ 303
Additions for tax positions taken in a prior year	—	—
Additions for tax positions taken in the current year	—	—
Reductions for tax positions taken in the prior year due to settlement	—	—
Reductions for tax positions taken in the prior year due to statutes lapsing	—	—
Gross unrecognized tax benefits at end of year	$ 303	$ 303

The uncertain tax positions giving rise to the unrecognized tax benefits of $0.3 million at December 31, 2024 and 2023 relate to the timing of certain income and deductions for federal income tax purposes taken by Histogenics prior to the Company's reverse merger with Histogenics. The reversal of unrecognized tax benefits would not have any impact on the effective tax rate in the future and is not expected to create cash liability.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In a normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. The Company's tax years are still open from 2019 to present.

13. Net Loss per Share of Common Stock

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2024 and 2023 (in thousands, except share and per share amounts): For purposes of earnings per share, the Series B Convertible Preferred shares have the same characteristics as common stock and have no liquidation or other material preferential rights over common stock and accordingly, have been considered as a second class of common stock in the computation of net loss per share regardless of their legal form. Losses are allocated between the common shares and the Series B Convertible Preferred Stock on a pro rata basis as they share equally in losses and residual net assets on an as-converted basis.

	Year ended December 31,	
	2024	2023
Net loss attributable to common shareholders— basic and diluted	(54,010)	(63,078)
Weighted shares used in calculating net loss per common share — basic and diluted	270,995,121	244,327,057
Net loss per share attributable to common shareholders — basic and diluted	$ (0.20)	$ (0.26)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	(44)	—
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	54,745	—
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	$ (0.80)	$ —

The following potentially dilutive securities have been excluded from the computation of diluted weighted-average shares outstanding, as their inclusion would have been antidilutive:

	Year ended December 31,	
	2024	2023
Stock options to purchase common stock	16,197,148	13,161,228
RSUs	1,902,457	2,982,661
PSUs	872,352	—
Warrants	628,725	628,834
Series B Convertible Preferred Stock (as converted to common stock)	—	547,450
Total	19,600,682	17,320,173

14. Commitments and Contingencies

Commitments

The Company has commitments under certain license and development agreements, lease agreements, commitments related to renovating an existing facility for GMP, and debt agreements. Commitments under certain license and development agreements include annual payments, payments upon the achievement of certain milestones, and royalty payments based on net sales of licensed products (see Note 3). Commitments under lease agreements are future minimum lease payments (see Note 6). Commitments under debt agreements are the future payment of principal and accrued interest under the EB-5 Loan Agreement and the Loan and Security Agreement (see Note 8). Additionally, the Company does not expect to fulfill any commitments under the amended Co-Development, Supply and Commercialization Agreement with Bharat Biotech as a result of the termination of the COVAXIN program.

Contingencies

In June 2021, a securities class action lawsuit was filed against the Company and certain of its agents in the United States District Court for the Eastern District of Pennsylvania ("Court") (Case No. 2:21-cv-02725) that purported to state a claim for alleged violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder, based on statements made by the Company concerning the announcement of the Company's decision to pursue the submission of a BLA for COVAXIN for adults ages 18 years and older rather than pursuing an EUA. In July 2021, a second securities class action lawsuit was filed against the Company and certain of its agents in the Court (Case No. 2:21-cv-03182) that also purported to state a claim for alleged violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder, based on the same statements as the first complaint. In March 2022, the Court consolidated these two related securities class action lawsuits and appointed a lead plaintiff. In March 2024, the Third Circuit affirmed the Court's decision to dismiss with prejudice the consolidated securities class action lawsuits.

In August 2021, a stockholder derivative lawsuit was filed derivatively on behalf of the Company against certain of its agents and the nominal defendant Ocugen in the Court (Case No. 2:21-cv-03876) that purported to state a claim for breach of fiduciary duty and contribution for violations of Sections 10(b) and 21(d) of the Exchange Act, based on facts and circumstances relating to the securities class action lawsuits and seeking contribution and indemnification in connection with claims asserted in the securities class action lawsuits. In September 2021, a second stockholder derivative lawsuit was filed derivatively on behalf of the Company against certain of its agents and the nominal defendant Ocugen in the Court (Case No. 2:21-cv-04169) that purported to state a claim for breach of fiduciary duties, unjust enrichment, abuse of control, waste of corporate assets, and contribution for violations of Sections 10(b) and 21(d) of the Exchange Act, based on the same allegations as the first complaint. The parties to both stockholder derivative lawsuits stipulated to the consolidation of the two stockholder derivative lawsuits. In April 2024, the Court dismissed without prejudice the consolidated stockholder derivative lawsuits.

In April 2024, a securities class action lawsuit was filed against the Company and certain of its agents in the Court (Case No. 2:24-cv-01500) that purported to state a claim for alleged violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder, based on statements made by the Company concerning the Company's previously-issued audited consolidated financial statements for each fiscal year beginning January 1, 2020 and its previously-issued unaudited condensed consolidated financial statements for each of the first three quarters in such years and the effectiveness of the Company's disclosure controls and procedures during each such period. The complaint seeks unspecified damages, interest, attorneys' fees, and other costs. In October 2024, the lead plaintiff filed an amended complaint, and in December 2024, the Company filed a motion to dismiss. In February 2025, the lead plaintiff filed an opposition to the motion to dismiss, and the Company intends to file a reply in support of the motion to dismiss by March 10, 2025.

In May 2024, a stockholder derivative lawsuit was filed on behalf of the Company against certain of its agents and the nominal defendant Ocugen in the Court (Case No. 2:24-cv-02234) that purported to state a claim for breach of fiduciary duties, unjust enrichment, abuse of control, gross mismanagement, waste of corporate assets, violations of Section 14(a) of the Exchange Act,

and contribution for violations of Sections 10(b) and 21(d) of the Exchange Act, based on the facts and circumstances relating to the securities class action and seeking damages and certain governance reforms in connection with claims asserted in the securities class action. In June 2024, the Court approved the parties' joint stipulation for an order staying the derivative lawsuit pending resolution of a motion to dismiss in the related securities class action. In the third quarter of 2024, four additional stockholder derivative lawsuits were filed on behalf of the Company against certain of its agents and the nominal defendant Ocugen in the Court (Case Nos. 2:24-cv-03119, 2:24-cv-03209, 2:24-cv-04813, 2:24-cv-04864) asserting similar facts and claims as the first complaint, and in January 2025, a stockholder derivative lawsuit was filed on behalf of the Company against certain of its agents and the nominal defendant Ocugen in the Delaware Court of Chancery (Case No. 2025-0095) asserting similar facts and claims related to breaches of fiduciary duty, unjust enrichment and insider trading.

The Company believes that the lawsuits are without merit and intends to vigorously defend against them. At this time, no assessment can be made as to their likely outcome or whether the outcome will be material to the Company. No information is available to indicate that it is probable that a loss has been incurred and can be reasonably estimated as of the date of the consolidated financial statements and, as such, no accrual for the loss has been recorded within the consolidated financial statements.

15. **Segment Reporting**

The Company has one operating and reportable segment relating to the research, development and commercialization of its novel gene and cell therapies and vaccines. The segment derives its current revenue from a co-development and commercialization agreement with CanSinoBIO. The Company does not track expenses on an individual program basis for overhead costs, as the Company utilizes its resources across all programs.

The Company's Chief Operating Decision Maker (the "CODM"), its Chief Executive Officer, manages the Company's operations on an integrated basis for the purposes of allocating resources. When evaluating the Company's financial performance, the CODM reviews financial information at the consolidated level. The CODM uses net loss as the measure of profit or loss to allocate resources and assess performance. The CODM regularly reviews net loss as reported on the Company's consolidated statements of operations and comprehensive loss. Financial forecasts and budget to actual results used by the

CODM to assess performance and allocate resources, as well as those used for strategic decisions related to headcount and capital expenditures are also reviewed on a consolidated basis.

The measure of segment assets is reported on the balance sheet as total assets.

The table below is a summary of the segment profit or loss, including significant segment expenses (in thousands):

	Year ended December 31,	
	2024	2023
Collaborative arrangement revenue	$ 4,055	$ 6,036
Less:		
OCU400	6,846	7,318
OCU410 and OCU410ST	3,653	4,029
NeoCart	489	1,336
COVAXIN	25	8,824
Inhaled mucosal vaccine platform	2,464	629
OCU200	379	713
Unallocated costs:		
Research and development personnel costs	12,992	13,868
Facilities and other support costs	2,984	1,507
Other [(a)]	2,294	1,349
Total research and development	32,126	39,573
General and administrative	26,686	31,994
Total operating expenses	58,812	71,567
Loss from operations	(54,757)	(65,531)
Other income (expense), net	703	2,453
Segment and consolidated net loss	$ (54,054)	$ (63,078)

[(a)] Other expenses include travel for research and development and a clinical and regulatory data repository system for each program.

16. **Revision to Previously Reported Unaudited Financial Statements**

As previously disclosed in Company's Quarterly Report on Forms 10-Q for each of the quarterly and year to date periods ended September 30, 2024, the Company concluded to revise its financial statements to treat the Series B Convertible Preferred Stock as a second class of common stock for purposes of presenting earnings per share. In accordance with the guidance in ASC 260, equity classified shares of stock need to be assessed to determine if they are preferred stock or are in substance common shares and therefore represent a second class of common shares. The rights of the Series B Convertible Preferred Stock were substantially equivalent to common shares and they did not have any material preferential rights compared to common shares. As such, the Company concluded that it should have considered the Series B Convertible Preferred Stock to be a second class of common stock for purposes of presenting earnings per share under the two-class method. Additionally, for the three and six months ended June 30, 2024, the Company previously reduced the net loss attributable to common shareholders by a deemed dividend of $4,988 thousand related to the reacquisition by the Company of the Series B Convertible Preferred Stock. This adjustment should not have been made as the guidance for adjusting the numerator to reflect the implicit gain or loss upon the re-acquisition of shares is only applicable to shares which are substantively considered preferred stock for earnings per share purposes. The impact of these errors is immaterial to the previously filed financial statements. Within our previously filed Quarterly Report on Form 10-Q for the period ended September 30, 2024, the Company revised earnings per share to separately present loss per share for common stock and for the Series B Convertible Preferred Stock and also removed the deemed dividend which previously reduced the net loss attributable to common shareholders for the three and six months ended June 30, 2024. The Company has not revised and will not revise any periods prior to January 1, 2024 because the impact to those periods is immaterial. The 2024 revisions will be reflected in the comparative 2024 periods in future filings where those periods are included with appropriate disclosures.

The impacts of the revision to the previously reported, unaudited condensed consolidated financial statements are summarized within the tables below.

Impact of the revision for the period ended March 31, 2024 **(in thousands, except share and per share amounts) (Unaudited)**

	Three months ended March 31, 2024 (as previously reported)	Adjustment	Three months ended March 31, 2024 (as adjusted)
Net loss	$ (11,924)	—	(11,924)
Net loss attributable to common shareholders— basic and diluted	(11,924)	25	(11,899)
Weighted shares used in calculating net loss per common share — basic and diluted	257,232,636	—	257,232,636
Net loss per share attributable to common shareholders — basic and diluted	(0.05)		(0.05)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	—	(25)	(25)
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	—	54,745	54,745
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	—		(0.46)

Impact of the revision for the periods ended June 30, 2024 **(in thousands, except share and per share amounts) (Unaudited)**

	Three months ended June 30, 2024 (as previously reported)	Adjustment	Three months ended June 30, 2024 (as adjusted)
Net loss	$ (15,280)	—	(15,280)
Reacquired Series B Convertible Preferred Stock	4,988	(4,988)	—
Net loss attributable to common shareholders— basic and diluted	(10,292)	(4,967)	(15,259)
Weighted shares used in calculating net loss per common share — basic and diluted	257,353,857	—	257,353,857
Net loss per share attributable to common shareholders — basic and diluted	(0.04)		(0.06)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	—	(21)	(21)
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	—	54,745	54,745
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	—		(0.38)

	Six months ended June 30, 2024 (as previously reported)	Adjustment	Six months ended June 30, 2024 (as adjusted)
Net loss	$ (27,204)	—	(27,204)
Reacquired Series B Convertible Preferred Stock	4,988	(4,988)	—
Net loss attributable to common shareholders— basic and diluted	(22,216)	(4,941)	(27,157)
Weighted shares used in calculating net loss per common share — basic and diluted	257,293,247	—	257,293,247
Net loss per share attributable to common shareholders — basic and diluted	(0.09)		(0.11)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	—	(47)	(47)
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	—	54,745	54,745
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	—		(0.86)