UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2023
OR

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

For the transition period from to

Commission File Number: 001-36829

Rocket Pharmaceuticals, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware **(State or Other Jurisdiction of Incorporation or Organization)**	**04-3475813** **(IRS Employer Identification No.)**
9 Cedarbrook Drive, Cranbury, NJ **(Address of Principal Executive Offices)**	**08512** **(Zip Code)**

(609) 659-8001
(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, $0.01 par value	RCKT	NASDAQ Global 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 Exchange 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 Exchange Act). Yes ☐ No ☒

The aggregate market value of the common stock held by non-affiliates of the registrant as of June 30, 2023 (the last business day of the registrant's most recently completed second fiscal quarter) was approximately $1.2 billion, based upon the closing price on the NASDAQ Global Market reported for such date.

As of February 22, 2024, there were 90,504,248 shares of common stock, $0.01 par value per share, outstanding.

Documents Incorporated by Reference

Part III of this annual report on Form 10-K incorporates by reference information (to the extent specific sections are referred to herein) from the registrant's definitive proxy statement for its 2024 Annual Meeting of Stockholders (the "Proxy Statement"). The Proxy Statement will be filed with the United States Securities and Exchange Commission within 120 days of the end of the period covered by this Annual Report on Form 10-K.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K ("Annual Report") contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Annual Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "develop," "estimate," "expect," "expand," "future," "hope," "intend," "likely," "may," "plan," "potential," "predict," "project," "pursue," "seek," "should," "strategy," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the U.S. Food and Drug Administration ("FDA");
- the timing of and our ability to submit regulatory filings, including filings with the FDA, and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates;
- our ability to acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances or joint ventures;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- the development of our direct manufacturing capabilities for our AAV programs;
- our ability to expand our pipeline to target additional indications that are compatible with our gene therapy technologies;
- our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future do business, including compliance with applicable regulatory requirements and laws;
- our ability to obtain and enforce patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- our estimates regarding our capital requirements; and
- our ability to obtain additional financing and raise capital as necessary to fund operations or pursue business opportunities.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Annual Report.

Any forward-looking statements in this Annual Report reflect our current views with respect to future events or to our future financial performance and 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 these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part I, Item 1A. Risk Factors and elsewhere in this Annual Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events, or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. Unless stated otherwise, references in this Annual Report to "us," "we," "our," or our "Company" and similar terms refer to Rocket Pharmaceuticals, Inc.

SUMMARY OF THE MATERIAL RISKS ASSOCIATED WITH OUR BUSINESS

Our business is subject to numerous risks and uncertainties that you should be aware of in evaluating our business. These risks and uncertainties include, but are not limited to, the following:

- If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates, we may not be successful in commercializing those product candidates if and when they are approved.
- If we fail to obtain necessary additional funding to conduct our planned research and development efforts, we could be forced to delay, reduce, or eliminate our product development programs or commercial development efforts.
- We have never generated any revenue from product sales and may never be profitable.
- We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.
- If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit, or terminate planned clinical trials, the occurrence of any of which would harm our business, financial condition, results of operations and prospects.
- Preliminary, interim or topline results in our ongoing clinical studies may not be indicative of results obtained when these studies are completed and success in early clinical studies may not be indicative of results obtained in later studies.
- Our product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.
- Our gene therapy product candidates are based on novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.
- Even if we successfully complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.
- We may never obtain approval for any of our product candidates in the United States (“U.S.”) or the European Union (“EU”), or other jurisdictions, which would limit our ability to realize our full market potential.
- Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory obligations and continued regulatory scrutiny.
- If approved, our product candidates may face competition from biosimilars approved through an abbreviated regulatory pathway.
- Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.
- If we are successful in commercializing any product, our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm and diminished profits and future earnings.
- We are subject to stringent laws, rules, regulations, policies, industry standards and contractual obligations regarding data privacy and security and may be subject to additional related laws and regulations in jurisdictions into which we expand.
- We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.
- We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.
- We have limited experience in manufacturing, and there can be no assurance that we will be able to successfully manufacture products.
- Our manufacturing facilities are subject to significant government regulations and approvals, which are often costly and could result in adverse consequences to our business if we fail to comply with the regulations or maintain the approvals.
- Product liability lawsuits against us could cause us to incur substantial liabilities and could limit the potential commercialization of any products that we may develop.
- Our ability to successfully develop and commercialize our product candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.
- The commercial success of any of our product candidates will depend upon the degree of market acceptance of gene therapy by physicians, patients, third-party payors and others in the medical community.
- Ethical, legal, and social issues may reduce demand for any gene therapy products for which we obtain marketing approval.
- We may not be successful in our efforts to expand our pipeline of additional product candidates.
- The success of our research and development activities, clinical testing, and commercialization, upon which we primarily focus, is uncertain.
- We expect to rely on third parties to conduct some or all aspects of our drug product manufacturing, research, and

preclinical and clinical testing, and these third parties may not perform satisfactorily.

- Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved, or commercialized in a timely manner or at all, which could negatively impact our business.
- Our rights to intellectual property for the development and commercialization of our product candidates are subject to the terms and conditions of licenses granted to us by others.
- If we are unable to obtain and maintain patent protection for our products and related technology or are unable to otherwise protect our intellectual property rights and trade secrets related to our product candidates, we may not be able to compete effectively in our markets.
- Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.
- If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.
- We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.
- Our business could suffer if it loses the services of, or fails to attract, key personnel.
- We may need to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.
- Our employees, principal investigators, consultants, and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading which could harm our business.
- Given our commercial relationships outside of the U.S., in particular the EU, a variety of risks associated with international operations could harm our business.
- We may fail to realize the anticipated benefits of potential acquisitions or business combinations.
- Future formations of strategic alliances or joint ventures with third parties could disrupt our business and harm our financial condition and operating results.
- If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.
- RTW Investments, LP, our largest stockholder, may have the ability to significantly influence matters submitted to stockholders for approval.
- Future sales of our common stock in the public market could cause the market price of our common stock to drop significantly, even if our business is performing well.
- 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, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.
- Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our development programs.
- Unfavorable national or global economic conditions or political developments could adversely affect our business, financial condition or results of operations.

The summary risk factors described above should be read together with the text of the full risk factors below and in the other information set forth in this Annual Report, including our consolidated financial statements and the related notes, as well as in other documents that we file with the Securities and Exchange Commission ("SEC"). If any such risks and uncertainties actually occur, our business, prospects, financial condition, and results of operations could be materially and adversely affected. The risks summarized above or described in full elsewhere in this Annual Report are not the only risks that we face. Additional risks and uncertainties not currently known to us, or that we currently deem to be immaterial may also materially adversely affect our business, prospects, financial condition, and results of operations.

PART I

Item 1. Business

Overview

We are a fully integrated, late-stage biotechnology company focused on the development of first, only and best in class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. We have three clinical-stage *ex vivo* lentiviral vector ("LV") programs, which include programs for:

- Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells;
- Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction; and
- Pyruvate Kinase Deficiency ("PKD"), a red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia.

In September 2023, the FDA accepted the Biologics License Application ("BLA") and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 studies were completed in 2023 with regulatory filings in the United States ("U.S.") and Europe ("EU") for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., we also have two clinical stage and one pre-clinical stage *in vivo* adeno-associated virus ("AAV") programs, which include programs for:

- Danon disease ("DD"), a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The DD program is currently in an ongoing Phase 2 trial.
- Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), an inheritable cardiac disorder that is characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. This program received FDA clearance of an Investigational New Drug ("IND") application and we have initiated a Phase 1 study.
- BAG3 Dilated Cardiomyopathy ("DCM"), which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. Our program utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Gene Therapy Overview

Genes are composed of sequences of deoxyribonucleic acid ("DNA"), which provide the code for proteins that perform a broad range of physiologic functions in all living organisms. Although genes are passed on from generation to generation, genetic changes, also known as mutations, can occur in this process. These changes can result in the lack of production of proteins or the production of altered proteins with reduced or abnormal function, which can in turn result in disease.

Gene therapy is a therapeutic approach in which an isolated gene sequence or segment of DNA is administered to a patient, most commonly for the purpose of treating a genetic disease that is caused by genetic mutations. Currently available therapies for many genetic diseases focus on administration of large proteins or enzymes and typically address only the symptoms of the disease. Gene therapy aims to address the disease-causing effects of absent or dysfunctional genes by delivering functional copies of the gene sequence directly into the patient's cells, offering the potential for curing the genetic disease, rather than simply addressing symptoms.

We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic) genes are removed and are replaced with a functional form of the missing or mutant gene that is the cause of the patient's genetic disease. The functional form of a missing or mutant gene is called a therapeutic gene, or the "transgene." The process of inserting the transgene is called "transduction." Once a virus is modified by replacement of the viral genes with a transgene, the modified virus is called a "viral vector." The viral vector delivers the transgene into the targeted tissue or organ (such as the cells inside a patient's bone marrow). We have two types of viral vectors in development, LV and AAV. We believe that our LV and AAV-based programs have the potential to offer a significant and long-lasting therapeutic benefit to patients.

The gene therapies can be delivered either (1) *ex vivo* (outside the body), in which case the patient's cells are extracted and the vector is delivered to these cells in a controlled, safe laboratory setting, with the modified cells then being reinserted into the patient, or (2) *in vivo* (inside the body), in which case the vector is injected directly into the patient, either intravenously ("IV") or directly into a specific tissue at a targeted site, with the aim of the vector delivering the transgene to the targeted cells.

We believe that scientific advances, clinical progress, and the greater regulatory acceptance of gene therapy have created a promising environment to advance gene therapy products as these products are being designed to restore cell function and improve clinical outcomes, which in many cases include prevention of death at an early age. The FDA approval of several gene therapies in recent years indicates that there is a regulatory pathway forward for gene therapy products.

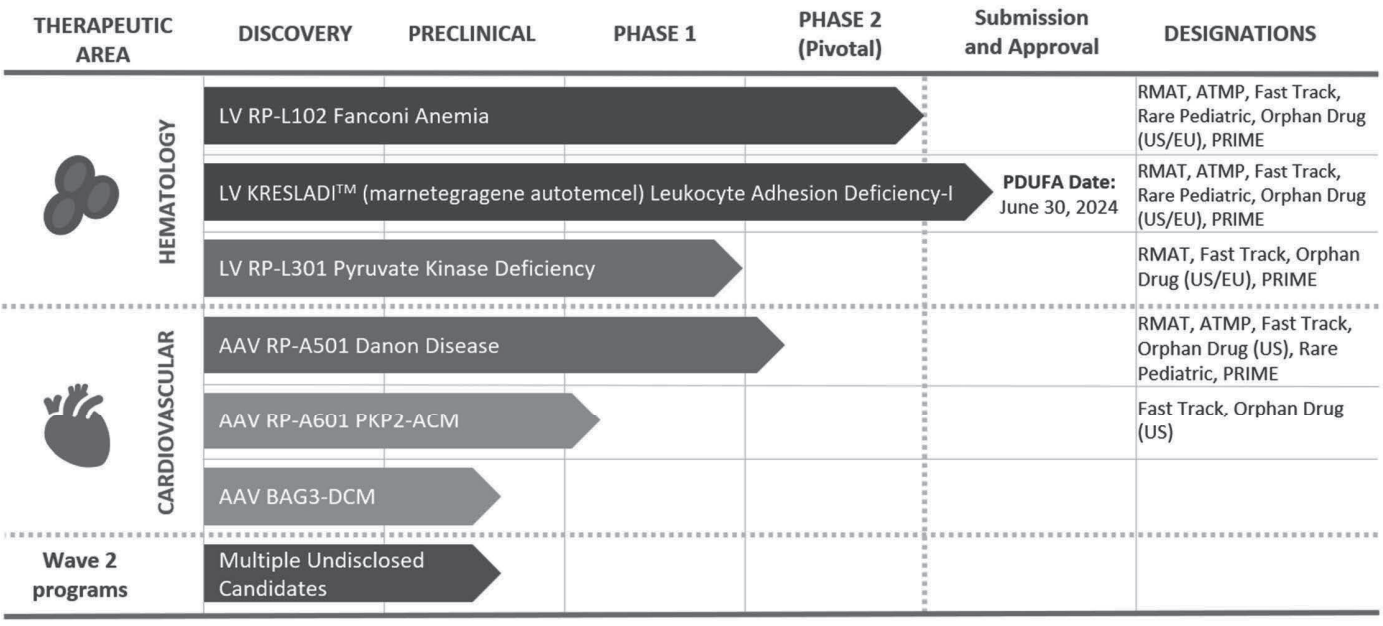
Essential Terminology

Set forth below is an abbreviated index of certain key terms and optimal ranges of values used in the discussion of LV and AAV gene therapies.

Term	Definition	Optimal Ranges
	LV Therapy (hematopoietic disorders)	
CD34+ cell(s)	Hematopoietic Stem Cell (most CD34+ cells are not true stem cells, but this continues to be the most clinically useful measure)	Will depend on underlying disorder, generally > 1 million CD34+ cells/kg.
Vector copy number (VCN) [product]	The average number of gene copies per infused stem cell (as determined by DNA analysis; this is an average ratio, not a precise value)	0.5 to 2 has been target in some LV clinical studies (5.0 generally considered maximum)
Vector copy number (VCN) [*in vivo*, post-treatment]	The average number of gene copies per peripheral blood or bone marrow cell (as determined by DNA analysis; this is an average ratio, not a precise value)	Will depend on underlying disorder, but many disorders may be correctable with *in vivo* VCNs << 1.0
	AAV Therapy	
Vector copy number (VCN) [*in vivo*, post-treatment]	The average number of gene copies per cell in the organ of interest (as determined by DNA analysis; this is an average ratio, not a precise value)	Will depend on underlying disorder, but *vivo* VCNs << 1.0

Pipeline Overview

The chart below shows the current phases of development of our programs and product candidates:



Cardiovascular Programs

Danon Disease

DD is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. DD is caused by mutations in the gene encoding lysosome-associated membrane protein 2 ("LAMP-2"), a mediator of autophagy. This mutation results in the accumulation of autophagic vacuoles, predominantly in cardiac and skeletal muscle. Male patients often require heart transplantation and typically die in their teens or twenties from progressive heart failure. Along with severe cardiomyopathy, other DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of congestive heart failure ("CHF") are not believed to modify progression to end-stage CHF. Patients with end-stage CHF may undergo heart transplant, which currently is available to a minority of patients, is associated with significant short- and long-term complications and is not curative of the disorder in the long-term. RP-A501 is in clinical trials as an *in vivo* therapy for DD, which is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the EU.

DD is an X-linked dominant, monogenic rare inherited disorder characterized by progressive cardiomyopathy which is almost universally fatal in males even in settings where cardiac transplantation is available. DD predominantly affects males early in life and is characterized by absence of *LAMP2B* expression in the heart and other tissues. Preclinical models of DD have demonstrated that AAV-mediated transduction of the heart results in reconstitution of *LAMP2B* expression and improvement in cardiac function.

We currently have one AAV program targeting DD, RP-A501. We have treated seven patients in the RP-A501 Phase 1 clinical trial, which enrolled adult/older adolescent and pediatric male DD patients. This includes a first cohort evaluating a low-dose (6.7e13 genome copies (gc)/kilogram (kg)) in adult/older adolescent patients aged 15 or greater (n=3), a second cohort evaluating a higher dose (1.1e14 gc/kg) in adult/older adolescent patients aged 15 or greater (n=2), and a pediatric cohort at a low dose level (6.7e13 gc/kg; n=2).

As previously disclosed, a patient receiving therapy on the high dose cohort (1.1e14 gc/kg dose) had progressive heart failure and underwent a heart transplant at month five following therapy. This patient had more advanced disease than the four other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished baseline left ventricle ejection fraction (35%) on echocardiogram and markedly elevated left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

Based on the initial efficacy observed in the low dose cohort and to mitigate complement-mediated safety concerns observed in the high dose cohort (thrombotic microangiopathy ("TMA")) and in agreement with the FDA, we are focusing on the low dose (6.7e13 gc/kg) and we will no longer administer doses of 1.1e14 gc/kg or higher in this trial. Additional safety measures have been implemented and are reflected in the updated trial protocol. These measures include exclusion of patients with end-stage heart failure, and a refined immunomodulatory regimen involving transient B- and T-cell mediated inhibition, with emphasis on preventing complement activation, while also enabling lower steroid doses and earlier steroid taper, with all immunosuppressive therapy discontinued 2-3 months following administration of RP-A501.

We conducted a variety of efficacy assessments in the Phase I clinical study to measure the prospect of benefit for patients. These assessments included the following:

- New York Heart Association ("NYHA") Functional Classification is the most commonly used heart failure classification system. NYHA Class II is where a patient exhibits a slight limitation of physical activity, is comfortable at rest, and ordinary physical activity results in fatigue, palpitation and/or dyspnea. Class I is where a patient exhibits no limitation of physical activity and ordinary physical activity does not cause undue fatigue, palpitation and/or dyspnea. Class III and IV are considered more severe or advanced heart failure.
- Brain natriuretic peptide ("BNP") is a blood-based evaluation and a key marker of heart failure with prognostic significance in CHF and cardiomyopathies. Elevations in BNP are strongly associated with worsening heart failure and poor outcomes in cardiovascular disease.
- High sensitivity troponin I ("hsTnI") is a blood-based evaluation and a key marker of cardiac injury, one that is (like BNP) frequently elevated in DD patients and has been shown to be markedly elevated in patients with advanced stage disease.
- Echocardiographic measurements of heart thickness, most notably, left ventricular mass and maximal left ventricular wall thickness, indicate the degree of hypertrophy present in the heart.
- Kansas City Cardiovascular Questionnaire ("KCCQ") is a validated, patient-reported outcomes assessment that measures a patients perception of their heart failure symptoms, impact of disease on physical and social function, and the impact of their heart failure on overall health status and quality of life. Assessment scores range from 0 (very poor health status) to 100 (excellent health status). Changes in KCCQ score of +/- 5 points are considered meaningful and have been shown to correlate with outcomes.

- Histologic examination of endomyocardial biopsies via hematoxylin and eosin ("H&E") histology and electron microscopy is used to detect evidence of DD-associated tissue derangements, including the presence of autophagic vacuoles and disruption of myofibrillar architecture, each of which are characteristic of DD-related myocardial damage.
- LAMP2B gene expression in endomyocardial biopsy samples is measured via both immunohistochemistry and Western blot and confirms the presence of LAMP2B protein in DD cardiac tissue following RP-A501 treatment.

On January 9, 2023, we presented positive efficacy updates from our Phase I study of RP-A501 during the 41st Annual J.P. Morgan Healthcare Conference. The data presented included several additional months of follow-up, which showed further improvements in key biomarkers, echocardiographic and functional measures. A summary of these updates is provided in the table below. We also provided additional natural history comparator data, which showed the marked divergence of the course of Phase I patients from that of untreated patients in terms of key biomarkers (BNP) and functional measures (NYHA Class). Furthermore, RP-A501 continued to be well tolerated at 2-3 years post treatment in both adult/older adolescent high and low-dose cohorts and at 8 to 13 months in the pediatric cohort. In the pediatric cohort, no significant immediate or delayed toxicities, significant skeletal myopathy, or late transaminase elevation have been observed.

Improvement or Stabilization Observed Across Key Biomarker, Echo Findings and Functional Measures in Phase 1 RP-A501 study

Cohort	Patient ID	Most recent visit (months)	Δ hsTnI	Δ BNP	Δ LV mass	Δ LV max wall thickness	Δ NYHA class	Δ KCCQ score
Low dose pediatric	1008	12	↓86%	↓83%	↓29%[1]	↓15%[1]	II -> I	+32.3
	1009	6	↓90%	↓62%	↓21%	↑3%	II -> I	+26
Low dose adult/ adolescent	1001	36	↓98%	↑8%	↓32%	↓9%	II -> II[2]	+5.3
	1002	36	↓96%	↓94%	↓48%	↓40%	II -> I	+17.8
	1005	30	↓46%	↑6%	↓14%	↓27%	II -> I	+8.3[3]
High dose adult/ adolescent	1006	24	↓63%	↓69%	↓27%	↓15%	II -> I	+3.1

Darker Green = improved; Lighter Green = minimal change (stabilization)
Does not include pt 1007 in Ph1 trial who had advanced HF with EF<40% at enrollment and received HTx 5M following tx due to pre-existing advanced HF. Patient is currently stable. Data cut-off September 27, 2022.
[1] Patient 1008 echocardiographic parameters are M9 visit (M12 pending).
[2] Patient 1002 NYHA class depicted for M30 visit (M36 pending).
[3] Patient 1005 KCCQ score depicted for M24 visit (M30 pending).

In addition to these clinical updates, we also provided updates on our in-house manufacturing activities. As of January 2023, we had successfully produced 2 cGMP RP-A501 batches that have superior specifications to Phase I material in both titer and full versus empty particles. We believe the improved quality of our in-house manufactured product will allow for full dosing with lower total viral particles, potentially further optimizing the safety profile of RP-A501. Furthermore, we have agreement from the FDA on the continued utilization of HEK-293 cell-based process through commercialization as well as our comparability approach and potency assay.

In May 2023, we presented previously disclosed results from the Phase I study of RP-A501 at the American Society of Gene & Cell Therapy ("ASGCT") 26th Annual Meeting, As of the most recent data extraction, all six patients that remain in follow-up continued to show signs of improvement or stabilization.

Results from the ongoing Phase 1 DD trial represent one of the most comprehensive investigational gene therapy datasets for any cardiac condition. RP-A501 was generally well tolerated with evidence of durable treatment activity and improvement of DD for both pediatric patients with up to nine months of follow-up and four adult/older adolescent patients with up to 36 months of follow-up. All adult/older adolescent and pediatric patients who received a closely monitored immunomodulatory regimen showed improvements across tissue, laboratory, and imaging-based biomarkers, as well as in NYHA class (from II to I) and KCCQ scores with follow-up of six to 36 months.

On September 12, 2023, we announced that alignment was reached with the FDA on the global Phase 2 pivotal trial of RP-A501 for DD. The global, single-arm, multi-center Phase 2 pivotal trial will evaluate the efficacy and safety of RP-A501 in 12 patients with DD, including a pediatric safety run-in (n=2), with a natural history comparator and a dose level of 6.7 x 1013 GC/kg.

- To support accelerated approval, the study will assess the efficacy of RP-A501 as measured by the biomarker-based co-primary endpoint consisting of improvements in LAMP2 protein expression (≥ Grade 1, as measured by immunohistochemistry), and reductions in left ventricular mass.
- Key secondary endpoint is change in troponin. Additional secondary endpoints will include natriuretic peptide, KCCQ, NYHA class event free survival to 24 months and treatment emergent safety events. These endpoints could support full approval with longer-term follow-up.
- A global natural history study will serve as an external comparator and run concurrently to the Phase 2 pivotal trial.
- In-house manufacturing has been completed with sufficient high-quality drug product produced to fully supply the Phase 2 pivotal study. Potency assays have been developed and qualified in accordance with FDA guidance.

We have filed Clinical Trial Application ("CTA") and Investigational Medicinal Product Dossier ("IMPD") for RP-A501 with the relevant Member States through the EU Clinical Trial Information System ("CTIS") and the Medical and Healthcare Products Regulatory Agency ("MHRA"). We are working towards initiation of Phase 2 pivotal trial activities in Europe and the UK.

Recently Achieved Milestones

On February 7, 2023, we announced that RP-A501 received regenerative medicine advanced therapy ("RMAT") designation from the FDA, and on May 31, 2023, we received priority medicines ("PRIME") designation from the European Medicines Agency ("EMA"). On September 12, 2023, we announced our alignment with the FDA on our pivotal study design for RP-A501 in DD and we have initiated the global study.

Plakophilin-2 Arrhythmogenic Cardiomyopathy (PKP2-ACM)

Arrhythmogenic cardiomyopathy ("ACM") is an inheritable cardiac disorder that is characterized by a high propensity for arrhythmias and sudden death, a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, and fibrofatty replacement of the myocardium. Most commonly, the cardiomyopathy initially manifests in the right ventricular free wall, so the disease was termed arrhythmogenic right ventricular dysplasia/cardiomyopathy (ARVD/ARVC). However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term ACM. Mutations in the PKP2 gene comprise the most frequent genetically identified etiology of familial ACM. PKP2 encodes for the protein Plakophilin-2, which is a component of the desmosome, an intercellular complex involved in cell-cell adhesion. PKP2 is also involved in transcriptional regulation of calcium signaling between cardiomyocytes. Patients with mutations in PKP2 are typically heterozygous and demonstrate reduced expression of PKP2 in the myocardium. Mean presentation is at the age of 35, and patients have a very high lifetime risk of ventricular arrhythmias, structural ventricular abnormalities, and sudden cardiac death ("SCD").

There are no specific available medical therapies available that have been shown to be highly effective for ACM, and current treatment protocols follow standard ventricular arrhythmia and cardiomyopathy guidelines, which involve lifestyle modifications (i.e. exercise limitation) and include drug treatments such as beta blockers, anti-arrhythmics and diuretics. The use of these therapies is driven by the arrhythmia burden and severity of cardiomyopathy. These therapies do not modify the course of the disease, and generally provide only symptomatic and/or palliative support. Upon diagnosis, a substantial percentage of patients receive an implantable cardiac defibrillator ("ICD") for primary or secondary prevention of ventricular arrhythmias and SCD. Of note, ICDs are not curative, and breakthrough life-threatening arrythmias may persist with ongoing risk of death. Furthermore, ICDs do not prevent the progression to end-stage heart failure. ICD firings, although lifesaving, are physically and emotionally traumatic events. Patients whose condition progresses to end-stage heart failure are considered for cardiac transplantation which, while curative of underlying disease, is itself associated with significant morbidity and mortality. Hence there exists a high unmet medical need in this population. PKP2-ACM is estimated to have a prevalence of 50,000 patients in the US and EU.

We currently have one AAV program targeting PKP2-ACM, RP-A601, which is a recombinant AAVrh.74 vector expressing PKP2a. PKP2-ACM is typically caused by heterozygous pathogenic mutations in the PKP2 gene resulting in reduced PKP2 expression in the myocardium. A once-administered gene therapy that addresses the root cause of the disease (PKP2 deficiency) early in the disease course, could mitigate the early electrical remodeling and diminish the risk of life-threatening arrhythmias and SCD associated with ACM, potentially impeding the development of irreversible cardiac structural changes. Prevention of syncopal episodes, life-threatening arrythmias, SCD, ICD shocks and the resulting anxiety, discomfort and hospitalizations is anticipated to result in a vastly improved quality of life and survival benefit. Furthermore, such an approach could spare patients the need for lifelong adherence to multiple arrhythmia and heart failure drugs that are nonspecific for PKP2-ACM and are associated with their own side effects, enabling patients an opportunity to live without exercise restrictions and with diminished concern for arrhythmias, palpitations, ICD shocks and progression to end-stage heart failure.

In May 2023, we presented preclinical efficacy data for RP-A601 at the American Society of Gene and Cell Therapy 26th Annual meeting. Nonclinical studies conducted by the Sponsor, RP-A601 have demonstrated efficacy in altering the natural history of PKP2-driven ACM. 100% of PKP2 cKO animals treated with the study drug exhibited extended survival to the longest timepoint measured (5 months), reduced cardiac dilation and fibrofatty replacement/fibrosis of the myocardium, preserved left ventricular function, and mitigation of the arrhythmic phenotype. Untreated PKP2 cKO mice had a median survival of approximately one month. These results were published in January 2024 in the journal Circulation: Genomic and Precision Medicine.

We have initiated a multi-center Phase 1 study for RP-A601. The multi-center Phase 1 dose escalation trial will evaluate the safety and preliminary efficacy of RP-A601 in at least six adult PKP2-ACM patients with ICDs and overall high risk for arrhythmias. The study will assess the impact of RP-A601 on PKP2 myocardial protein expression, cardiac biomarkers, and clinical predictors of life-threatening ventricular arrhythmias and sudden cardiac death. Patients in the dose-escalation trial will receive a single dose of RP-A601. The starting dose will be 8×10^{13} GC/kg.

Recently Achieved Milestones

We have achieved pre-clinical proof-of-concept for RP-A601 in an animal model representative of PKP2-ACM, completed pharmacology and GLP toxicology studies, produced GMP drug product, and developed an appropriate potency assay to support a Phase I study. On May 9, 2023, we announced FDA clearance of the IND, and on June 8, 2023, we announced receipt of FDA Fast Track and Orphan Drug Designations. We have since initiated the U.S. Phase 1 study.

BAG3 Dilated Cardiomyopathy

Dilated cardiomyopathy ("DCM") is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. A familial association of DCM can be identified in 20-50% of DCM patients, with up to 40% of familial patients having an identifiable genetic cause. Mutations in the BAG3 gene (BCL-2-associated athanogene 3) are among the more common pathogenic genetic variants observed in familial DCM and these variants are highly penetrant, with approximately 80% of individuals with disease-causing genetic variants in the BAG3 gene developing DCM at > 40 years of age. BAG3 protein is associated with a variety of cellular functions including cardiac contractility, protein quality control (as a co-chaperone), cardiomyocyte structural support and anti-apoptosis. BAG3 associated dilated cardiomyopathy (BAG3-DCM) leads to early onset, rapidly progressing heart failure and significant mortality and morbidity. We estimate that the prevalence of BAG3-associated DCM in the U.S. to be as many as 30,000 individuals.

Currently, DCM patients with a BAG3 mutation are treated with the standard of care for heart failure, which include angiotensin converting enzyme inhibitors, angiotensin receptor blockers, neprilysin inhibitors, beta-adrenergic receptor antagonists, or beta-blockers, aldosterone antagonists and/or diuretics, along with certain lifestyle changes, and do not address the underlying cause of disease. Patients who meet specific parameters may also undergo placement of an implantable cardioverter defibrillator, a cardiac resynchronization device or a combination of the two. There is no current therapy directly targeting the underlying mechanism of BAG3 associated DCM, and patients diagnosed with BAG3 associated DCM appear to progress to end-stage heart failure and death more rapidly than patients with DCM not associated with BAG3 variants. For example, approximately 19% of patients with BAG3-DCM require mechanical cardiac support, heart transplant, or have heart failure related death at 12 months after diagnosis, nearly twice the rate of similarly staged non-BAG3-DCM patients.

In December 2022, we completed our acquisition of Renovacor, Inc. ("Renovacor") which provided Rocket with Renovacor's recombinant AAV9-based gene therapy program designed to deliver a fully functional BAG3 gene to augment BAG3 protein levels in cardiomyocytes and slow or halt progression of BAG3-DCM. Initial proof of concept for AAV9-BAG3 has been demonstrated in studies of BAG3-knockout mouse models, which show treated mice have improved ejection fraction versus untreated knockout mice and comparable ejection fraction to walk test controls at timepoints 4- and 6-weeks post injection.

Recently Achieved Milestones

We are in the process of evaluating the optimal development pathway for this program and plan to submit an IND for BAG3-DCM in 2024.

Hematology Programs

Fanconi Anemia Complementation Group A (FANCA)

FA, a rare and life-threatening DNA-repair disorder, generally arises from a mutation in a single FA gene. An estimated 60% to 70% of cases arise from mutations in the Fanconi-A ("FANCA") gene, which is the focus of our program. FA results in bone marrow failure, developmental abnormalities, myeloid leukemia, and other malignancies, often during the early years and decades of life. Bone marrow aplasia, which is bone marrow that no longer produces any or very few red and white blood cells and platelets leading to infections and bleeding, is the most frequent cause of early morbidity and mortality in FA, with a median onset before 10 years of age. Leukemia is the next most common cause of mortality, ultimately occurring in about 20% of patients later in life. Solid organ malignancies, such as head and neck cancers, can also occur, although at lower rates during the first two to three decades of life.

Although improvements in allogeneic (donor-mediated) hematopoietic stem cell transplant ("HSCT"), currently the most frequently utilized therapy for FA, have resulted in frequent hematologic correction of the disorder, HSCT is associated with both acute and long-term risks, including transplant-related mortality, graft failure, and graft versus host disease, a sometimes fatal side effect of allogeneic transplant characterized by painful ulcers in the GI tract, liver toxicity and skin rashes, as well as increased risk of subsequent cancers. Our gene therapy program in FA is designed to enable a minimally toxic hematologic correction using a patient's own stem cells early in the disease course and administered without conditioning. We believe that the development of a broadly applicable autologous gene therapy can be transformative for these patients.

Each of our hematology programs utilize third-generation, self-inactivating LV to correct defects in patients' HSCs, which are the cells found in bone marrow that are capable of generating blood cells over a patient's lifetime. Defects in the genetic coding of HSCs can result in severe, and potentially life-threatening anemia, which is when a patient's blood lacks enough properly functioning red blood cells to carry oxygen throughout the body. Stem cell defects can also result in severe and potentially life-threatening decreases in white blood cells resulting in susceptibility to infections, and in platelets responsible for blood clotting, which may result in severe and potentially life-threatening bleeding episodes. Patients with FA have a genetic defect that prevents the normal repair of genes and chromosomes within blood cells in the bone marrow, which frequently results in the development of bone marrow failure, acute myeloid leukemia, and myeloid dysplastic syndrome types of blood cancers. FA patients also typically present with congenital defects. The average lifespan of an FA patient is estimated to be 30 to 40 years. The prevalence of FA in the U.S. and EU is estimated to be approximately 4,000 patients in total. In light of the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now believed to be 400 to 500 patients collectively in the U.S. and EU.

We currently have one *ex vivo* LV-based program targeting FA, RP-L102. RP-L102 is our lead LV-based program that we in-licensed from Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas ("CIEMAT"), which is a leading research institute in Madrid, Spain. Our Phase 2 registrational enabling clinical trials treating FA patients with RP-L102 at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford"), Great Ormond Street Hospital ("GOSH") in London and Hospital Infantil de Nino Jesus ("HNJ") in Spain completed treatment. The trial has treated a total of 12 patients from the U.S. and EU. Two additional patients were treated in the US Phase 1 study at Stanford such that a total of 14 patients have received RP-L102 on Rocket-sponsored clinical trials. Patients receive a single intravenous infusion of RP-L102 that utilizes fresh cells and "Process B" which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product.

Resistance to mitomycin-C, a DNA damaging agent, in bone marrow stem cells at a minimum time point of one year post treatment is the primary endpoint for our ongoing Phase 2 study. Per agreement with the FDA and EMA, engraftment leading to bone marrow restoration exceeding a 10% mitomycin-C resistance threshold could support a marketing application for approval.

In December 2022, we presented positive clinical data for RP-L102 at the 64th Annual Meeting of ASH. RP-L102 conferred phenotypic correction in at least six of 10 evaluable patients with ≥12 months of follow-up as demonstrated by increased resistance to MMC in bone marrow derived colony forming cells, concomitant genetic correction and hematologic stabilization. A seventh patient has displayed evidence of progressively increasing genetic correction as demonstrated by peripheral blood and bone marrow VCN's, with recent development of MMC resistance and possible indicators of hematologic stability after 36 months of follow-up. The primary endpoint has been achieved, based on a trial protocol in which statistical and clinical significance requires a minimum of five patients to attain increased MMC resistance at least 10% above baseline at two or more timepoints and concomitant evidence of genetic correction and clinical stabilization. The safety profile of RP-L102 has been highly favorable, and the treatment, administered without any cytotoxic conditioning, has been well tolerated. No signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102 have been observed.

We had previously disclosed that one of the initial five patients in this trial who had evidence of engraftment developed a T-cell lymphoblastic lymphoma approximately 22 months after RP-L102 administration. A surgical biopsy of the lymphoma indicated negligible gene markings (VCN of 0.003) at a juncture when concomitant VCN in blood and bone marrow were 0.26 and 0.42 respectively. These findings conclusively indicate that the lymphoma did not result from a LV-mediated insertion, as there were essentially no gene markings in the tumor (the very low but detectable VCN is likely the result of blood cells in the tumor specimen). FA is a cancer-predisposition syndrome and cancers may develop in patients under the age of 10. Importantly, the patient tolerated induction chemotherapy for the lymphoma without significant complications and is currently in a complete response. The presence of gene-corrected hematopoietic cells may have contributed to this patient's overall tolerance of chemotherapy.

In May 2023, we presented updated clinical data for RP-L102 at the ASGCT 26th Annual Meeting. As of the data cut-off (April 17, 2023), RP-L102 conferred sustained genetic correction in eight of 12 evaluable patients and comprehensive phenotypic correction in seven of 12 evaluable patients with ≥12 months of follow up as demonstrated by increased resistance to mitomycin-C (MMC) in bone marrow-derived colony forming cells and hematologic stabilization. The safety profile of RP-L102 continues to be highly favorable with no signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102. Polyclonal integration patterns have been observed in each of the seven patients with phenotypic, genetic, and hematologic evidence of engraftment. Pivotal trial enrollment and treatment have been completed.

Anticipated Milestones

Product filings for RP-L102 are anticipated in the first half of 2024 in the U.S. and Europe, and we are finalizing the Chemistry, Manufacturing, and Controls ("CMC") package with the FDA.

Leukocyte Adhesion Deficiency-I (LAD-I)

LAD-I is a rare autosomal recessive disorder of white blood cell adhesion and migration, resulting from mutations in the *ITGB2* gene encoding for the Beta-2 Integrin component, CD18. Deficiencies in CD18 result in an impaired ability for neutrophils (a subset of infection-fighting white blood cells) to leave blood vessels and enter tissues where these cells are needed to combat infections. As is the case with many rare diseases, accurate estimates of incidence are difficult to confirm; however, several hundred cases have been reported to date. Most LAD-I patients are believed to have the severe form of the disease. Severe LAD-I is notable for recurrent, life-threatening infections and substantial infant mortality in patients who do not receive an allogeneic HSCT. Mortality for severe LAD-I has been reported as 60 to 75% by age two in the absence of allogeneic HCST.

We currently have one *ex vivo* program targeting LAD-I, RP-L201. RP-L201 is a clinical program that we in-licensed from CIEMAT. University of California, Los Angeles ("UCLA") and its Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research is serving as the lead U.S. clinical research center for the registrational clinical trial for LAD-I, and HNJ and GOSH are serving as the lead clinical sites in Spain and London, respectively. This study has received a $6.6 million CLIN2 grant award from the California Institute for Regenerative Medicine ("CIRM") to support the clinical development of gene therapy for LAD-I.

The open-label, single-arm, Phase 1/2 registration-enabling clinical trial of RP-L201 has treated nine severe LAD-I patients to assess the safety and tolerability of RP-L201. The first patient was treated at UCLA with RP-L201 in the third quarter of 2019. Enrollment is now complete in both the Phase 1 and 2 portions of the study; nine patients have received RP-L201 at 3 investigative centers in the U.S. and Europe.

In December 2022, we presented positive clinical data at the 64th Annual Meeting of ASH. The presentation included previously disclosed top-line data at three to 24 months of follow-up after RP-L201 infusion for all patients and overall survival data for seven patients at 12 months or longer after infusion. We observed 100% overall survival at 12 months post-infusion via Kaplan Meier estimate and a statistically significant reduction in all hospitalizations, infection and inflammatory-related hospitalizations and prolonged hospitalizations for all nine LAD-I patients with three to 24 months of available follow-up. All patients, aged three months to nine years, demonstrated sustained CD18 restoration and expression on more than 10% of neutrophils (range: 20%-87%, median: 56%). Data also shows evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities. The safety profile of RP-L201 has been highly favorable in all patients with no RP-L201-related serious adverse events to date. Adverse events related to other study procedures, including busulfan conditioning, have been previously disclosed and consistent with the tolerability profiles of those agents and procedures.

In May 2023, at the ASGCT 26th annual meeting, we presented updated top-line data at 12 to 24 months of follow-up for all nine patients in our Phase 1/2 clinical trial showing 100% overall survival at 12 months post-infusion. All patients continue to demonstrate evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities, and the safety profile of RP-L201 remains highly favorable with follow-up of 12-36 months. No evidence of replication-competent lentivirus has been observed. Insertion site analyses indicate highly polyclonal integration patterns across the entire cohort.

Recently Achieved and Anticipated Milestones

A BLA filing for RP-L201 was accepted by the FDA with priority review in October of 2023 with an initial Prescription Drug User Fee Act ("PDUFA") date of March 31, 2024. On February 13, 2024, the review time was extended by three months, to June 30, 2024, to allow additional time to review clarifying CMC information submitted by Rocket in response to FDA information requests. The FDA has further confirmed that an advisory committee meeting is not needed.

Pyruvate Kinase Deficiency (PKD)

Red blood cell PKD is a rare autosomal recessive disorder resulting from mutations in the pyruvate kinase L/R ("PKLR") gene encoding for a component of the red blood cell ("RBC") glycolytic pathway. PKD is characterized by chronic non-spherocytic hemolytic anemia, a disorder in which RBCs do not assume a normal spherical shape and are broken down, leading to decreased ability to carry oxygen to cells, with anemia severity that can range from mild (asymptomatic) to severe forms that may result in childhood mortality or a requirement for frequent, lifelong RBC transfusions. The pediatric population is the most commonly and severely affected subgroup of patients with PKD, and PKD often results in splenomegaly (abnormal enlargement of the spleen), jaundice and chronic iron overload which is likely the result of both chronic hemolysis and the RBC transfusions used to treat the disease. The variability in anemia severity is believed to arise in part from the large number of diverse mutations that may affect the PKLR gene. Estimates of disease incidence have ranged between 3.2 and 51 cases per million in the white U.S. and EU population. Industry estimates suggest at least 2,500 cases in the U.S. and EU have already been diagnosed. Market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 patients per year.

We currently have one *ex vivo* LV-based program targeting PKD, RP-L301. RP-L301 is a clinical stage program that we in-licensed from CIEMAT.

We are conducting a global Phase 1 open-label, single-arm, clinical study has enrolled 2 adult patients and 2 pediatric patients (age 8-17) in the U.S. and Europe and is intended to assess the safety, tolerability, and preliminary activity of RP-L301. Stanford serves as the lead site in the U.S. for adult and pediatric patients, HNJ serves as the lead site in Europe for pediatrics, and Hospital Universitario Fundación Jiménez Díaz serves as the lead site in Europe for adult patients. Both adult and pediatric enrollment is completed in the Phase 1 study.

In May 2023, we presented positive updated clinical data at the ASGCT 26th Annual Meeting (data cut-off May 3, 2023), which included up to 30 months of follow-up from the two treated adult patients and early clinical data from the first pediatric patient treated with RP-L301. Robust and sustained efficacy was observed in both adult patients at up to 30 months post-infusion evidenced by normalized hemoglobin (from baseline pre-treatment levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, and red blood cell transfusion independence. Furthermore, both adult patients reported improved quality of life with documented improvements via formal quality of life assessments. The safety profile continues to appear highly favorable, with no RP-L301-related serious adverse events in either of the adult patients. Insertion site analyses in peripheral blood and bone marrow in both adult patients through 24 months post-RP-L301 demonstrated highly polyclonal patterns and there has been no evidence of insertional mutagenesis. The first pediatric patient infusion of RP-L301 was well tolerated, with engraftment achieved at day +15, hospital discharge less than one month following infusion, no RP-L301 related serious adverse events and early signs of efficacy. There were no red blood cell transfusion requirements following engraftment. Both adult and pediatric enrollment is completed in the Phase 1 study.

In October 2023, we presented positive updated clinical data at the 30th Annual Congress at ESGCT (data cut-off October 9, 2023), including up to 36 months of follow-up in the adult cohort and more limited follow-up of 6 months in the pediatric cohort. Sustained efficacy has been demonstrated in adult cohort including hemoglobin normalization, transfusion independence, decreased hemolysis, and quality of life improvement; hemoglobin improvement relative to pre-treatment baseline has been observed in pediatric cohort. The safety profile remains favorable.

Recently Achieved Milestones

In early 2023, we announced receipt of FDA RMAT and EMA PRIME designation for RP-L301 based on the robust efficacy observed in the Phase 1 treated patients.

We have reached agreement with FDA on study design of Phase 2 pivotal trial of RP-L301. Based on positive safety and efficacy data from the Phase 1 study, we have aligned with the FDA on the pivotal study design to support accelerated approval and are initiating a 10-patient, single-arm Phase 2 pivotal trial with a primary endpoint of ≥1.5 point Hgb improvement at 12 months.

cGMP Manufacturing

Our 103,720 square foot manufacturing facility in Cranbury, New Jersey has been scaled up to manufacture AAV drug product for our Phase 2 pivotal study in DD. The facility also houses lab space for research & development and quality. We reached an understanding with the FDA on chemistry, manufacturing, and controls requirements to start AAV cGMP manufacturing at our in-house facility as well as potency assay plans for a Phase 2 pivotal trial in DD.

Strategy

We seek to bring hope and relief to patients with devastating, undertreated, rare pediatric diseases through the development and commercialization of potentially curative first in class gene therapies. To achieve these objectives, we intend to develop into a fully-integrated biotechnology company. In the near and medium-term, we intend to develop our first in class product candidates, which are targeting devastating diseases with substantial unmet need, develop proprietary in-house analytics and manufacturing capabilities and continue to commence registration trials for our currently planned programs. In the medium and long-term, pending favorable data, we expect to submit BLAs for the rest of our suite of clinical programs, and establish our gene therapy platform and expand our pipeline to target additional indications that we believe to be potentially compatible with our gene therapy technologies. In addition, during that time, we believe that our currently planned programs will become eligible for priority review vouchers from the FDA that provide for expedited review. We have assembled a leadership and research team with expertise in cell and gene therapy, rare disease drug development and product approval.

We believe that our competitive advantage lies in our disease-based selection approach, a rigorous process with defined criteria to identify target diseases. We believe that this approach to asset development differentiates us as a gene therapy company and potentially provides us with a first-mover advantage.

Intellectual Property

We strive to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets relating to our proprietary technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy that may be important for the development of our business. We additionally intend to rely on regulatory protection afforded through orphan drug designations, data exclusivity, market exclusivity, and patent term extensions where available.

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

We have developed and in-licensed numerous patents and patent applications and possess substantial know-how and trade secrets relating to the development and commercialization of gene therapy products. Our proprietary intellectual property, including patent and non-patent intellectual property, is generally directed to gene expression vectors and methods of using the same for gene therapy. As of February 22, 2024, our patent portfolio includes both owned and in-licensed patent families relating to our product candidates and related technologies, discussed more fully below.

Fanconi Anemia

Our FA patent portfolio includes granted patents in Australia, Japan, and Russia and pending applications in the U.S., Europe, Japan, China and other countries with claims directed to polynucleotide cassettes and expression vector compositions containing FA complementation group genes and methods for using such vectors to provide gene therapy in mammalian cells for treating FA. These applications were exclusively in-licensed from CIEMAT, Centro de Investigacion Biomedica En Red, (“CIBER”), Fundacion Instituto de investigacion Sanitaria Fundacion Jimenez Diaz, (“FIISFJD”), and Fundacion Para la Investigacion Biomedica del Hospital Del Nino Jesus. We expect any patents in this family, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2037, absent any patent term adjustments or extensions.

Pyruvate Kinase Deficiency

Our PKD patent portfolio includes granted patents in Europe, China, Hong Kong, Japan, Mexico, South Korea, Australia, India, Russia, Singapore, and the U.S. and a pending patent application in the U.S., EU, Japan, China and other countries with claims directed to polynucleotide cassettes and expression vector compositions containing pyruvate kinase genes and methods for using such vectors to provide gene therapy in mammalian cells for treating pyruvate kinase deficiency. These applications are exclusively in-licensed from CIEMAT, CIBER, and FIISFJD. We expect any patents in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2037-2038, absent any patent term adjustments or extensions.

Danon Disease

Our DD patent portfolio includes both proprietary intellectual property and a patent family in-licensed from the University of California, San Diego, which includes granted patents in Europe, India, the U.S., and Hong Kong, allowed patent applications in Japan and Russia, and pending patent applications in the U.S., Europe, Japan, China and other countries with claims directed to the treatment of DD. We expect any patents in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2037 absent any patent term adjustments or extensions. We also own granted patents in the U.S. and Russia and pending patent applications in the U.S., Europe, Japan, China and other countries with claims directed to gene therapy vectors for the treatment of DD; the U.S. patent issued in 2020. Any patents, if issued, arising from these patent applications, are expected to expire in 2039, absent any patent term adjustments or extensions, if the appropriate maintenance, renewal, annuity, or other governmental fees are paid. We have also filed additional patent applications directed to methods for treatment of DD. Any patents, if issued, arising from these patent applications, are expected to expire in 2040-2041, absent any patent term adjustments or extensions, if the appropriate maintenance, renewal, annuity, or other governmental fees are paid.

Leukocyte Adhesion Deficiency

Our patent portfolio includes pending patent applications in the U.S., EU, Japan, China and other countries with claims directed to transduction of allogeneic HSCT, which may be relevant to our LAD-I program. We expect any patents arising from these patent applications, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2039, absent any patent term adjustments or extensions.

Future Objectives

Our objective is to continue to expand our portfolio of patents and patent applications in order to protect our gene therapy product candidates and manufacturing processes. From time to time, we may also evaluate opportunities to sublicense our portfolio of patents and patent applications that we own or exclusively license, and we may enter into such licenses from time to time. The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the U.S., a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office ("USPTO") in granting a patent or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

The term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration of a U.S. patent as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug was under regulatory review. 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 one patent applicable to an approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the filing of a BLA, we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and third parties. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of its premises and physical and electronic security of its information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Material Contracts

License Agreements with CIEMAT

In March 2016, we entered into a license agreement with CIEMAT, CIBER, and FIISFJD, (collectively, "CIEMAT"), granting us worldwide, exclusive rights to certain patents, know-how and other intellectual property relating to LVs containing the human PKLR gene solely within the field of treating PKD. Under the terms of the agreement, we are obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, we are obligated to pay CIEMAT an up-front payment, royalty payments based on net sales of products or processes involving any of the licensed intellectual property, developmental and regulatory milestone payments, and sublicense revenue payments. We are responsible for prosecuting and maintaining the licensed patents at our expense, in cooperation with CIEMAT. We also have the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with CIEMAT. For five years following the effective date of the license agreement, we had a right of first refusal to license any improvements to the licensed intellectual property obtained by CIEMAT at market value. We are obligated to license (without charge) to CIEMAT for non-commercial use any improvements to the licensed intellectual property that we create.

As consideration for the licensed rights, we paid CIEMAT an initial upfront license fee of €0.03 million (approximately $0.03 million) which was expensed as research and development ("R&D") costs. We are obligated to make aggregate milestone payments of up to €1.4 million (approximately $1.5 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the PKD license, we are obligated to pay a low to mid-single digit percentage royalty on net sales, subject to specified adjustments, by us or our sublicensees or affiliates. In the event that we enter into a sublicense agreement with a sublicensee, we will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

We may terminate this agreement at any time by providing CIEMAT with 90 days advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

In July 2016, we entered into a license agreement with CIEMAT granting us worldwide, exclusive rights to certain patents, know-how, data and other intellectual property relating to LVs containing the FANCA gene solely within the field of human therapeutic uses of VSV-G packaged integration component LVs for FA type-A gene therapy. This license is only sublicensable with the prior consent of CIEMAT, not to be unreasonably withheld. Under the terms of the agreement, we are obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, we are obligated to pay CIEMAT an up-front payment, royalty payments based on net sales of products or processes involving any of the licensed intellectual property, regulatory and financing milestone payments, and sublicense revenue payments. We are responsible for prosecuting and maintaining the licensed patents at our expense, in cooperation with CIEMAT. We also have the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with CIEMAT. For five years following the effective date of the license agreement, we have a right of first refusal to license any improvements to the licensed intellectual property obtained by CIEMAT at market value. We are obligated to license (without charge) any improvements to the licensed intellectual property that we create to CIEMAT for non-commercial use.

As consideration for the licensed rights, we paid CIEMAT an initial upfront license fee of €0.1 million (approximately $0.1 million), which was expensed as R&D costs. We are obligated to make aggregate milestone payments of up to €5.0 million (approximately $6.0 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the license, we are obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by us or our sublicensees or affiliates. In the event that we enter into a sublicense agreement with a sublicensee, we will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

We may terminate this agreement at any time by providing CIEMAT with 90 days' advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

License Agreement for LAD-I with CIEMAT and UCLB

We entered into a license agreement in November 2017, effective September 2017, with CIEMAT and UCL Business PLC ("UCLB", and collectively with CIEMAT, "Licensors"), granting us worldwide, exclusive rights to certain patents, know-how and other intellectual property relating to LVs containing the human LAD-I gene solely within the field of treating LAD-I. Under the terms of the agreement, we are obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public, (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, we are obligated to pay Licensors an up-front payment, royalty payments in the mid-single digit percentages based on net sales of products or processes involving any of the licensed intellectual property, developmental and regulatory milestone payments, and sublicense revenue payments. We are responsible for prosecuting and maintaining the licensed patents at our expense, in cooperation with Licensors. We also have the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with Licensors. For five years following the effective date of the license agreement, we have a right of first refusal to license any improvements to the licensed intellectual property obtained by Licensors at market value. We are obligated to license (without charge) any improvements to the licensed intellectual property that we create to Licensors for non-commercial use.

As consideration for the licensed rights, we paid Licensors an initial upfront license fee of €0.03 million (approximately $0.04 million), which was expensed as R&D costs. We are obligated to make aggregate payments of up to €1.4 million (approximately $1.5 million) to Licensors upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the LAD-I license, we are obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by us or our sublicensees or affiliates. In the event that we enter into a sublicense agreement with a sublicensee, we will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

We may terminate this agreement at any time by providing Licensors with 90 days advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

License Agreement for DD with UCSD

In February 2017, we entered into a license agreement with The Regents of the University of California, represented by its San Diego campus ("UCSD"), under which UCSD granted us an exclusive, sublicensable, worldwide license to certain intellectual property rights for the treatment of lysosomal storage diseases, including DD. In exchange for the license, we became obligated to make an up-front payment, certain clinical and commercial milestone payments, royalty payments (on net sales of products covered by a valid claim within the licensed intellectual property), maintenance fees and sublicense revenue payments. We paid an upfront license fee of $0.05 million and are obligated to make aggregate milestone payments of up to $1.5 million to UCSD upon the achievement of specified development and regulatory milestones for the treatment of DD. A reduced schedule of milestone payments applies to achieving the same milestones for additional indications. With respect to any commercialized products covered by the agreement, we are obligated to pay a low single digit percentage royalty on net sales, subject to specified adjustments. If we enter into a sublicense agreement with a sublicensee, we will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances. We are also subject to certain diligence milestones for development of a product using the intellectual property licensed from UCSD under this agreement.

The term of the license agreement with UCSD is through the expiration of the licensed patents, some of which are still in the pending application phase.

REGENXBIO, Inc. License

On November 19, 2018, we entered into a license agreement with REGENXBIO Inc. ("RGNX"), pursuant to which we obtained an exclusive license for all U.S. patents and patent applications related to RGNX's NAV AAV-9 vector for the treatment of DD in humans by *in vivo* gene therapy using AAV-9 to deliver any known LAMP2 transgene isoforms and all possible combinations of LAMP2 transgene isoforms (the "Field"), as well as an exclusive option to license (the "Option Right") all U.S. patents and patent applications for two additional NAV AAV vectors in the Field (each, a "Licensed Patent" and collectively, the "Licensed Patents").

In consideration for the rights granted to us under the license agreement, we made an upfront payment to RGNX of $7.0 million which was expensed to R&D costs in the 2018 consolidated statement of operations. A fee of $2.0 million per additional vector would be due if we exercise our Option Right to purchase additional vectors. The license agreement provides for royalties payable to RGNX in the high-single digits to low-teens on net sales levels of products incorporating the Licensed Patents (the "Licensed Products") during the royalty term. If successful, we will be required to make milestone payments to RGNX of up to $13.0 million for each Licensed Product upon the achievement of specified clinical development and regulatory milestones in the U.S. and EU. In addition, we shall pay RGNX 20% of the payment fees received from a priority review voucher issued in connection with or otherwise related to a Licensed Product. These royalty obligations are subject to specified reductions if additional licenses from third parties are required. We must also pay RGNX a portion of all non-royalty sublicense income (if any) received from sublicensees. We paid a $1.0 million license fee payment under the RGNX agreement upon the dosing of the first DD patient in 2019 and a $2.0 million license fee payment upon initiation of a Phase 2 pivotal trial in 2023. There were no additional milestones achieved or related payments made during the years ended December 31, 2023 and 2022.

At-the-Market Offering Program

On February 28, 2022, the Company entered into a sales agreement (the "Sales Agreement"), with Cowen and Company, LLC ("Cowen"), with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock, par value $0.01 per share, having an aggregate offering price of up to $200 million (the "Shares") through Cowen as its sales agent.The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to our shelf registration statement on Form S-3. We filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. We will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. We also agreed to provide Cowen with customary indemnification and contribution rights. We have reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through December 31, 2023, we sold 4.2 million shares under the at-the-market offering program for gross proceeds of $65.8 million, less commissions of $2.0 million for net proceeds of $63.8 million. During the year ended December 31, 2023, we sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commissions of approximately $0.6 million for net proceeds of $17.2 million. On September 12, 2023, the Company and Cowen entered into an amendment pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million.

Competition

The biotechnology and pharmaceutical industries, including in the field of gene therapy, are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products and novel therapies. While we believe that our experience and scientific knowledge provides us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical and biotechnology companies, new market entrants and new technologies, as well as from academic institutions, government agencies and private and public research institutions, which may in the future develop products to treat the indications targeted by our pipeline that have not yet been conceived. Any product candidates that we successfully develop and commercialize will compete with existing therapies such as bone marrow transplantation and new therapies that may become available in the future. We believe that the key competitive factors affecting the success of our product candidates, if approved, are likely to be efficacy, safety, convenience, price, pharmaco-economic value, tolerability and the availability of coverage and adequate reimbursement from governmental authorities and other third-party payors. In addition, we intend to develop single treatment curative therapies for clinical indications that address mortality or high morbidity, which could differentiate us from potential competitors developing alternative competitive therapies that may require chronic or repetitive treatment.

Other early-stage companies may also compete 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 companies developing gene therapies. These companies also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

We anticipate that we will face intense and increasing competition as new drugs and therapeutic modalities enter the market and advanced technologies become available. Our commercial opportunity could be reduced or eliminated if our potential competitors develop and commercialize products that are safer, more effective, have fewer adverse effects, are more convenient or are less expensive than any products that we may develop. Our potential competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for our products.

Manufacturing

Our gene therapy platform has two main components: the production of LV and AAV vectors and the target cell transduction process, which results in drug product. We commenced GMP manufacturing at our facility in Cranbury, New Jersey in 2022. We plan to supplement our own direct manufacturing capabilities with third-party manufacturers for our AAV programs. For our LV programs, we currently rely on third-party manufacturers to produce the plasmids, vectors, cell banks and final drug product for our clinical trials. We manage such production with our vendors on a purchase order basis in accordance with applicable master service and supply agreements. We have long-term agreements with these manufacturers. Whenever possible, we procure materials from redundant and multiple sources to mitigate risk. If any of our existing third-party suppliers should become unavailable to us for any reason, we believe that there are a number of potential replacements, although we might experience a delay in our ability to obtain alternative suppliers. We also do not have any current contractual relationships for the manufacture of commercial supplies of our product candidates if they become registered. With respect to commercial production of our product candidates in the future, we plan to pursue multiple options including direct manufacturing as well as outsourcing production of the active pharmaceutical (drug substance) ingredients and final drug product manufacturing (drug product) to contract manufacturing organizations if these products are approved and registered for marketing authorization by the applicable regulatory bodies.

We expect to continue to develop drug candidates that can be produced in a cost-effective manner through direct manufacturing or at contract manufacturing facilities. Should a supplier or manufacturer on which we have relied to produce a product candidate provide us with a faulty product or such product is later recalled, or should we experience such problems for our own products produced through direct manufacturing, we would likely experience delays and additional costs, each of which could be significant.

Government Regulation

FDA Regulation and Marketing Approval

In the U.S., the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act ("FDCA"), and biologics under the Public Health Service Act, the regulations promulgated under both laws and other federal, state, and local statutes and regulations. Failure to comply with the applicable U.S. regulatory requirements at any time during the product development process, approval process or after approval may subject an applicant to administrative or judicial sanctions and non-approval of product candidates. These sanctions could include, among other things, the imposition by the FDA of a clinical hold on trials, the FDA's refusal to approve pending applications or related supplements, withdrawal of an approval, untitled or warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, restitution, disgorgement, civil penalties, or criminal prosecution. Such actions by government agencies could also require us to expend a large amount of resources to respond to the actions. Any agency or judicial enforcement action could have a material adverse effect on us.

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, approval, manufacture, distribution and marketing of pharmaceutical products. These agencies and other federal, state and local entities regulate R&D activities and the testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, post-approval monitoring, advertising, promotion, sampling and import and export of our products. Our drug candidates must be approved by the FDA as biologics through the BLA approval process applicable to gene therapy product candidates, before they may be legally marketed in the U.S.

Within the FDA, the FDA's Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products and has published guidance documents with respect to the development of these types of products. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs.

The process required by the FDA before a biologic may be marketed in the U.S. generally involves the following:

- completion of non-clinical laboratory tests, animal studies and formulation studies conducted according to Good Laboratory Practice ("GLP"), or other applicable regulations;
- submission of an IND, which allows clinical trials to begin unless FDA objects within 30 days;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug or biologic for its intended use or uses conducted in accordance with FDA regulations and Good Clinical Practices ("GCP"), which are international ethical and scientific quality standards meant to ensure that the rights, safety and well-being of trial participants are protected, and that the integrity of the data is maintained;
- preparation and submission to the FDA of a BLA;
- submission of a user fee for FDA review of the BLA;
- review of the product by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of pre-approval inspection of manufacturing facilities and clinical trial sites at which the product,

or components thereof, are produced to assess compliance with current Good Manufacturing Practice ("cGMP") requirements, and if applicable, the FDA's current Good Tissue Practice ("cGTP") requirements, and of selected clinical trial sites to assess compliance with GCP requirements; and

- FDA approval of a BLA which must occur before a biologic can be marketed or sold.

Preclinical Studies

Preclinical studies include laboratory evaluation of the purity and stability of the manufactured drug substance or active pharmaceutical ingredient and the formulated drug or drug product, as well as in vitro and animal studies to assess the safety and activity of the drug for initial testing in humans and to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data, or literature and plans for clinical studies, among other things, are submitted to the FDA as part of an IND.

Companies usually must complete some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the drug in commercial quantities in accordance with ("cGMP") requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality, and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

IND and Clinical Trials

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Prior to commencing the first clinical trial, an initial IND, which contains the results of preclinical testing along with other information, such as information about product chemistry, manufacturing and controls and a proposed protocol, must be submitted to the FDA. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA within the 30-day time period raises concerns or questions about the drug product or the conduct of the clinical trial and imposes a clinical hold. A clinical hold may also be imposed at any time while the IND is in effect. In such a case, the IND sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin or re-commence. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence or continue.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the U.S., certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of institutional biosafety committees ("IBCs"), as set forth in the National Institutes for Health ("NIH") Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Under the NIH Guidelines, recombinant and synthetic nucleic acids are defined as: (i) molecules that are constructed by joining nucleic acid molecules and that can replicate in a living cell (i.e., recombinant nucleic acids); (ii) nucleic acid molecules that are chemically or by other means synthesized or amplified, including those that are chemically or otherwise modified but can base pair with naturally occurring nucleic acid molecules (i.e., synthetic nucleic acids); or (iii) molecules that result from the replication of those described in (i) or (ii). Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

A sponsor who wishes to conduct a clinical trial outside the U.S. may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of a BLA or IND so long as the clinical trial is conducted in compliance with GCP, and the FDA is able to validate the data from the study through an onsite inspection if the agency deems it necessary.

A separate submission to the existing IND must be made for each successive clinical trial to be conducted during product development. Further, an independent Institutional Review Board ("IRB") for each site at which the clinical trial will be conducted must review and approve the clinical trial before it commences at that site. Informed written consent must also be obtained from each trial subject. Regulatory authorities, including the FDA, or IRB, or the sponsor, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk or that the clinical trial is not being conducted in accordance with FDA requirements. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization as to whether or not a trial may move forward at designated check points based on access to certain data from the trial and may recommend halting the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy.

Human clinical trials for BLA approval typically involve a three-phase process, although some phases may overlap or be combined. Phase 1, the initial clinical evaluations, consists of administering the drug and testing for safety and tolerated dosages and in some indications such as rare disease, as preliminary evidence of efficacy in humans. Phase 2 involves a study to evaluate the effectiveness of the drug for a particular indication and to determine optimal dosage and dose interval and to identify possible adverse side effects and risks in a larger patient group. When a product is found safe, and initial efficacy is established in Phase 2, it is then evaluated in Phase 3 clinical trials. Phase 3 trials consist of expanded multi-location testing for efficacy and safety to evaluate the overall benefit-to-risk index of the investigational drug in relationship to the disease treated. The results of preclinical and human clinical testing are submitted to the FDA in the form of a BLA for approval to commence commercial sales.

All clinical trials must be conducted in accordance with FDA regulations, GCP requirements and their protocols in order for the data to be considered reliable for regulatory purposes. Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Government regulation may delay or prevent marketing of product candidates or new drugs for a considerable period of time and impose costly procedures upon our activities.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to disclose the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved up to a maximum of two years. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

The BLA Approval Process

In order to obtain approval to market a drug in the U.S., a marketing application must be submitted to the FDA that provides data establishing to the FDA's satisfaction the safety and effectiveness of the investigational drug for the proposed indication. The application includes all relevant data available from pertinent non-clinical or preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators that meet GCP requirements.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the End-of-Phase 1 or 2, and before a BLA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice and for the sponsor and the FDA to reach agreement on the next phase of development.

The results of product development, non-clinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of a BLA requesting approval to market the product for its intended indication. The FDA reviews all BLAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may request additional information rather than accept a BLA for filing. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. The FDA has 60 days from its receipt of a BLA to conduct an initial review to determine whether the application will be accepted for filing based on the Agency's threshold determination that the application is sufficiently complete to permit substantive review. The FDA reviews a BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA has agreed to specific performance goals on the review of BLAs. Specifically, FDA under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, as amended, the FDA has 10 months, from the filing date, in which to complete its initial review of an original BLA and respond to the applicant, and six months from the filing date of an original BLA designated for priority review. The review process may be extended by the FDA for three additional months to consider certain late-submitted information or information intended to clarify information already provided in the submission. After the FDA completes its substantive review of a BLA, it will communicate to the sponsor that the drug will either be approved, or it will issue a complete response letter to communicate that the BLA will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, non-clinical or manufacturing data that must be received before the application can be approved, with no implication regarding the ultimate approvability of the application or the timing of any such approval, if ever. If or when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA may issue an approval letter. FDA has committed to reviewing such resubmissions in two to six months depending on the type of information included. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation, and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving a BLA, the FDA typically will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA may inspect one or more clinical sites to assure compliance with GCP. For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with the cGTPs. These are FDA regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue-based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the cGTP requirements is to ensure that cell and tissue-based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through appropriate screening and testing. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and often will request additional testing or information. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GCP, the FDA may determine the data generated by the clinical site should be excluded from the primary efficacy analyses provided in the BLA. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase 4 or post-approval trials may be made a condition to be satisfied for continuing drug approval. The results of Phase 4 trials can confirm the effectiveness of a product candidate and can provide important safety information. In addition, the FDA has authority to require sponsors to conduct post-marketing trials to specifically address safety issues identified by the agency. See "Post-Marketing Requirements" below.

The FDA also has authority to require a Risk Evaluation and Mitigation Strategy ("REMS"), from manufacturers to ensure that the benefits of a drug outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the BLA submission. The need for a REMS is determined as part of the review of the BLA. Based on statutory standards, elements of a REMS may include "Dear Doctor letters," a medication guide, more elaborate targeted educational programs, and in some cases distribution and use restrictions, referred to as elements to assure safe use ("ETASU"). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. These elements are negotiated as part of the BLA approval, and in some cases the approval date may be delayed. Once adopted, REMS are subject to periodic assessment and modification.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, manufacturing processes or facilities, require submission and FDA approval of a new BLA or BLA supplement before the change can be implemented. A BLA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing BLA supplements as it does in reviewing BLAs.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution or use, or post-marketing trial requirements. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in restrictions on the product, including safety labeling or imposition of a REMS, the requirement to conduct post-market studies or clinical trials or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain, regulatory approval for our products, or obtaining approval but for significantly limited use, would harm our business. In addition, we cannot predict what adverse governmental regulations may arise from future U.S. or foreign governmental action.

The Hatch-Waxman Amendments

Under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, a portion of a product's U.S. patent term that was lost during clinical development and regulatory review by the FDA may be restored by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of a BLA, plus the time between the submission date of a BLA and the approval of that application, provided that the sponsor acted with diligence. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended and the extension must be applied for prior to expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Market Exclusivity

The Affordable Care Act, or ACA, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or the BPCIA, which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-approved reference biological product. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical trial or trials. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

A reference biological product is granted four (4) and twelve (12) year exclusivity periods from the time of first licensure of the product. FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four (4) years after the date of first licensure of the reference product, and FDA will not approve an application for a biosimilar or interchangeable product based on the reference biological product until twelve (12) years after the date of first licensure of the reference product. "First licensure" typically means the initial date the particular product at issue was approved in the U.S. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity, or potency. Therefore, one must determine whether a new product includes a modification to the structure of a previously approved product that results in a change in safety, purity, or potency to assess whether the licensure of the new product is a first licensure that triggers its own period of exclusivity. Whether a subsequent application, if approved, warrants exclusivity as the "first licensure" of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

In addition, under the Orphan Drug Act, FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the U.S., or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the U.S. for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Competitors, however, may receive approval of different products for the same indication than that for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan medicinal product status in the EU has similar, but not identical, benefits.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the U.S. and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data.

Rare Pediatric Disease Designation and Priority Review Vouchers

Under the FDCA, the FDA incentivizes the development of drugs and biological products that meet the definition of a "rare pediatric disease," defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the U.S. or affects more than 200,000 in the U.S. and for which there is no reasonable expectation that the cost of developing and making in the U.S. a drug or biological product for such disease or condition will be received from sales in the U.S. of such drug or biological product. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biological product application after the date of approval of the rare pediatric disease drug or biological product, referred to as a priority review voucher ("PRV"). A sponsor may request rare pediatric disease designation from the FDA prior to the submission of its BLA. A rare pediatric disease designation does not guarantee that a sponsor will receive a PRV upon approval of its BLA. Moreover, a sponsor who chooses not to submit a rare pediatric disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Congress has extended the PRV program through September 30, 2024, with the potential for PRVs to be granted through September 30, 2026.

Expedited Development and Review Programs

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

Any product submitted to FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as regenerative medicine advanced therapy ("RMAT") designation, priority review and accelerated approval. To qualify for RMAT designation, the product candidate must be a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except for those regulated solely under Section 361 of the Public Health Service Act and part 1271 of Title 21, Code of Federal Regulations; is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and preliminary clinical evidence indicates that the product has the potential to address unmet medical needs for such disease or condition. A gene therapy product may meet the definition of a regenerative medicine therapy for purposes of RMAT designation. A BLA for a product candidate that has received RMAT designation may be eligible for priority review or accelerated approval through use of surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites. Benefits of RMAT designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate with RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence from clinical studies, patient registries, or other sources of real-world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

A product candidate including one that received Fast Track or RMAT designation is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious condition compared to available therapies. FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.

Additionally, a biologic product may be eligible for accelerated approval if it is designed to treat a serious or life-threatening disease or condition and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, FDA currently requires, unless otherwise informed by the agency, pre-approval of promotional materials intended for dissemination or publication within 120 days of marketing approval.

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

Post-Marketing Requirements

Following approval of a new product, a pharmaceutical company and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and recordkeeping activities, reporting to the applicable regulatory authorities of adverse experiences with the product, providing the regulatory authorities with updated safety and efficacy information, product sampling and distribution requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the drug's approved labeling, or off-label use, limitations on industry-sponsored scientific and educational activities and requirements for promotional activities involving the internet. Although physicians may, in their independent professional medical judgment, prescribe legally available drugs for off-label uses, manufacturers typically may not market or promote such off-label uses. Modifications or enhancements to the product or its labeling or changes of the site of manufacture are often subject to the approval of the FDA and other regulators, who may or may not grant approval or may include a lengthy review process.

Prescription drug advertising is subject to federal, state, and foreign regulations. In the U.S., the FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Drug Supply Chain Security Act and the Prescription Drug Marketing Act, both of which are part of the FDCA.

In the U.S., once a product is approved, its manufacturing is subject to comprehensive and continuing regulation by the FDA. The FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMP. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Additionally, manufacturers and other parties involved in the supply chain for prescription drug products must also comply with product tracking and tracing requirements and notify the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the U.S. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and quality assurance activities. BLA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms, and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by the FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such product or may result in restrictions on a product, manufacturer, or holder of an approved BLA, including, among other things, recall, withdrawal of the product from the market, or refusal of importation. In addition, the manufacturer and/or holder of an approved BLA are subject to annual product and establishment fees. These fees are typically increased annually.

The FDA also may require post-marketing testing, also known as Phase 4 testing, to monitor the effects of an approved product or place conditions on an approval via a REMS that could restrict the distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from the FDA, mandated corrective advertising or communications with doctors, withdrawal of approval, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and may also require the implementation of other risk management measures. New government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

Coverage and Reimbursement

Sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors, including government healthcare program administrative authorities, managed care organizations, private health insurers, and other entities. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products. Therefore, our products, once approved, may not obtain market acceptance unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products.

The process for determining whether a third-party payor will provide coverage for a drug product typically is separate from the process for setting the price of a drug product or for establishing the reimbursement rate that the payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, also known as a formulary, or otherwise subject it to a health technology assessment. In either case, payer coverage rules might exclude certain FDA-approved drugs for a particular indication. A decision by a third-party payor not to cover our product candidates could reduce physician utilization of our products once approved. Moreover, a third-party payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. 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 product development. Additionally, coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug product or service does not ensure that other payors will also provide coverage for the medical product or service or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately and will be a time-consuming process.

The containment of healthcare costs has become a priority of federal, state, and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payors are increasingly challenging the prices charged for drug products and medical services, examining the medical necessity, and reviewing the cost effectiveness of drug products and medical services, in addition to questioning safety and efficacy. If these third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after FDA approval or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit.

The American Recovery and Reinvestment Act of 2009 provided funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures will be made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidates, if any such product or the condition that it is intended to treat is the subject of a study. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our product candidates, once approved. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the EU do not follow price structures of the U.S. and generally tend to be significantly lower.

Anti-Kickback and False Claims Laws and Other Regulatory Matters

In the U.S., among other things, the research, manufacturing, distribution, sale and promotion of drug products and medical devices are potentially subject to regulation and enforcement by various federal, state and local authorities in addition to the FDA, including the Department of Justice, Centers for Medicare & Medicaid Services, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency, state Attorneys General and other state and local government agencies. Our current and future business activities, including for example, sales, marketing, and scientific/educational grant programs must comply with healthcare regulatory laws, as applicable, which may include the Federal Anti-Kickback Statute, the Federal False Claims Act, as amended, the privacy and security regulations promulgated under the Health Insurance Portability and Accountability Act ("HIPAA"), as amended, physician payment transparency laws, and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended, which requires special pricing to both the Veterans Administration and other Federal agencies, as well as to certain safety net providers, referred to as 340B covered entities. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage, and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The Federal Anti-Kickback Statute makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully, directly or indirectly, in cash or in kind, solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of, any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as Medicare or Medicaid. The term "remuneration" has been broadly interpreted to include anything of value. The Federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Additionally, the intent standard under the Federal Anti-Kickback Statute was amended by the Patient Protection and Affordable Care Act, as amended by the Health Care Education and Reconciliation Act (collectively, the "ACA"), to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, a claim including items or services resulting from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the Federal False Claims Act. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties, and exclusion from participation in federal healthcare programs. In addition, many states have adopted laws similar to the Federal Anti-Kickback Statute. Some of these state prohibitions apply to the referral of patients for healthcare services reimbursed by any insurer, not just federal healthcare programs such as Medicare and Medicaid. Due to the breadth of these federal and state anti-kickback laws, and the potential for additional legal or regulatory change in this area, it is possible that our future business activities, including our sales and marketing practices and/or our future relationships with physicians and the medical community might be challenged under anti-kickback laws, which could harm us.

Federal false claims and false statement laws, including the civil False Claims Act, prohibits any person or entity from, among other things, knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent. Although we would not submit claims directly to payors, manufacturers can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, knowingly providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state, and third-party reimbursement for our products, and the sale and marketing of our products, are subject to scrutiny under this law. For example, pharmaceutical companies have been found liable under the Federal Civil False Claims Act in connection with their off-label promotion of drugs. Penalties for a civil False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and, although the Federal False Claims Act is a civil statute, conduct that results in a False Claims Act violation may also implicate various federal criminal statutes. If the government were to allege that we were, or convict us of, violating these false claims laws, we could be subject to a substantial fine and may suffer a decline in our stock price. In addition, private individuals have the ability to bring actions under the Federal Civil False Claims Act and certain states have enacted laws modeled after the Federal False Claims Act.

Additionally, HIPAA created additional federal criminal statutes that prohibit, among other things, 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, fictitious, or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. For example, there are federal government price reporting laws, which require us to calculate and report complex pricing metrics in an accurate and timely manner to government programs. In addition, as discussed below, a similar federal requirement under the Physician Payments Sunshine Act, requires certain manufacturers to track and report to the federal government certain payments provided to physicians and teaching hospitals made in the previous calendar year, as well as certain ownership and investment interests held by physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors) and their immediate family members. These laws may affect our sales, marketing, and other promotional activities by imposing administrative and compliance burdens on us. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state and federal authorities. Effective January 1, 2022, these reporting obligations extend to include transfers of value made to certain non-physician providers such as physician assistants and nurse practitioners.

In addition, 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 their respective implementing regulations, including the Final Omnibus Rule published on January 25, 2013, imposes specified requirements relating to the privacy, security, and transmission of individually identifiable health information on certain types of individuals and organizations. Although we will not likely be a "covered entity" under this law, our customers may require us to become a "business associate" to them for various purposes, which will require that we make ourselves amenable to lawsuits for any data breaches we may incur. In addition, certain state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other and from HIPAA in significant ways and may not have the same effect, thus complicating compliance efforts.

The failure to comply with regulatory requirements subjects us to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in significant criminal, civil and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in federal healthcare programs, such as Medicare and Medicaid, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, refusal to allow us to enter into supply contracts, including government contracts, contractual damages, reputational harm, administrative burdens, 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.

We plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the law and program requirements to which we will or may become subject because we intend to commercialize products that could be reimbursed under a federal healthcare program and other governmental healthcare programs. However, we cannot guarantee that this program will work effectively with respect to every federal and state law at each and every moment where such compliance is necessary.

Changes in law or the interpretation of existing law could impact our business in the future by requiring, for example: (i) changes to our manufacturing or sales arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; (iv) increases in our governmental rebate liability; or (v) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Healthcare Legislative Reform

In both the U.S. and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, was enacted, which, among other things, subjected biologic products to potential competition by lower-cost biosimilars; addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; created a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (increased to 70% pursuant to the Bipartisan Budget Act of 2018, effective as of January 1, 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D (which has been repealed effective 2025); and provided incentives to programs that increase the federal government's comparative effectiveness research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted including:

- In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals for deficit reduction of at least $1.2 trillion for the years 2013 through 2021. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction, which triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of, up to 2% per fiscal year, and, due to subsequent legislative amendments, will remain in effect through 2030 unless Congress takes additional action. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030 unless additional action is taken by Congress.
- On April 13, 2017, CMS published a final rule that gives states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.
- On May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no

obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

- On May 23, 2019, CMS published a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At a federal level, President Biden signed an Executive Order on July 9, 2021 affirming the administration's policy to (i) support legislative reforms that would lower the prices of prescription drug and biologics, including by allowing Medicare to negotiate drug prices, by imposing inflation caps, and, by supporting the development and market entry of lower-cost generic drugs and biosimilars; and (ii) support the enactment of a public health insurance option. Among other things, the Executive Order also directs HHS to provide a report on actions to combat excessive pricing of prescription drugs, enhance the domestic drug supply chain, reduce the price that the Federal government pays for drugs, and address price gouging in the industry; and directs the FDA to work with states and Indian Tribes that propose to develop section 804 Importation Programs in accordance with the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, and the FDA's implementing regulations. FDA released such implementing regulations on September 24, 2020, which went into effect on November 30, 2020, providing guidance for states to build and submit importation plans for drugs from Canada. On September 25, 2020, CMS stated drugs imported by states under this rule will not be eligible for federal rebates under Section 1927 of the Social Security Act and manufacturers would not report these drugs for "best price" or Average Manufacturer Price purposes. Since these drugs are not considered covered outpatient drugs, CMS further stated it will not publish a National Average Drug Acquisition Cost for these drugs. If implemented, importation of drugs from Canada may materially and adversely affect the price we receive for any of our product candidates. Additionally, on November 30, 2020, HHS published a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe harbors were delayed and recent legislation imposed a moratorium on implementation of the rule until January 1, 2032. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the Biden administration may reverse or otherwise change these measures, both the Biden administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs.

Most recently, on August 16, 2022, President Biden signed the Inflation Reduction Act ("IRA") which provides for (i) the government to set or negotiate prices for select high-cost Medicare Part D (beginning in 2026) and Medicare Part B drugs (beginning in 2028) that are more than nine years (for small-molecule drugs) or 13 years (for biological products) from their FDA approval, (ii) manufacturers to pay a rebate for Medicare Part B and Part D drugs when prices increase faster than inflation beginning in 2022 for Medicare Part D and 2023 for Medicare Part B drugs, and (iii) Medicare Part D redesign which replaces the current coverage gap provisions and establishes a $2,000 cap for out-of-pocket limits costs for Medicare beneficiaries beginning in 2025, with manufacturers being responsible for 10% of costs up to the $2,000 cap and 20% after that cap is reached. Implementation of the IRA has occurred through informal guidance, but the results of that guidance on the market for drugs, including our products, remains uncertain.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological 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 the healthcare reform measures that have been adopted and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private third-party payors.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal, and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates.

European Union Drug Review and Approval

Clinical Trial Approval

In the EU, an applicant for authorization of a clinical trial must obtain prior approval from the national competent authority of the EU Member States in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial at a specific study site after the relevant independent ethics committee has issued a favorable opinion in accordance with the laws of the Member State(s) concerned. In April 2014, the EU adopted the new Clinical Trials Regulation (EU) No 536/2014, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022. It overhauls the system of approvals for clinical trials in the EU. Specifically, the new legislation, which is directly applicable in all EU Member States (meaning that no national implementing legislation in each EU Member State is required), aims at simplifying and streamlining the approval of clinical trials in the EU. For instance, the new Clinical Trials Regulation provides for a streamlined application procedure through the EU CTIS via a single-entry point (instead of submitting applications separately to each national competent authority and ethics committee in the Member States in which the trial will be conducted) and strictly defined deadlines for the assessment of clinical trial applications. The Clinical Trials Regulation also makes it more efficient for EU Member States to evaluate and authorize applications together, via the Clinical Trials.

The transitory provisions of the new Clinical Trials Regulation offer sponsors the possibility to choose between the requirements of the previous Clinical Trials Directive and the Clinical Trials Regulation if the request for authorization of a clinical trial is submitted in the year after the new Clinical Trials Regulation became applicable i.e. January 31, 2023. As of January 31, 2023, all applications need to be submitted under and in accordance with the Clinical Trial Regulation. If the sponsor chooses to submit under the Clinical Trials Directive, the clinical trial continues to be governed by the Directive and the relevant implementing legislation in each EU Member State, as required, until three years after the new Clinical Trials Regulation became applicable. If a clinical trial continues for more than three years after the Clinical Trials Regulation became applicable, the Clinical Trials Regulation will at that time begin to apply to the clinical trial. The implementation of the Clinical Trial Regulation may require us to take additional steps and procedures to ensure that our clinical trials comply with applicable laws.

Marketing Authorization

In the EU, medicinal products can only be commercialized after obtaining a marketing authorization. There are two types of marketing authorizations: (1) the centralized authorization, which is issued by the European Commission through the centralized procedure based on the opinion of the Committee for Medicinal Products for Human Use ("CHMP"), a body of the EMA, and which is valid throughout the entire territory of the European Economic Area, or EEA (comprising the EU Member States plus Norway, Iceland and Liechtenstein); and (2) national marketing authorizations, which is issued by the competent authorities of the Member States of the EU and only authorize marketing in that Member State's national territory and not the EEA as a whole, including the harmonized issuance of marketing authorizations in several Member States upon the initial application ("Decentralized Procedure") or subsequently after the issuance of the initial national marketing authorization ("Mutual Recognition Procedure") in accordance with the procedures set forth in Regulation (EC) 1234/2008.

The centralized procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, advanced therapy medicinal products (i.e., gene-therapy, somatic cell-therapy, and tissue-engineered medicines) and medicinal products containing a new active substance indicated for the treatment of HIV/AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune diseases and other immune dysfunctions and 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. Gene therapy products are a type of advanced therapy medicinal product ("ATMP") in the EU. The scientific evaluation of marketing authorization applications for ATMPs is primarily performed by a specialized scientific committee called the Committee for Advanced Therapies ("CAT"). The CAT prepares a draft opinion on the quality, safety, and efficacy of the ATMP which is the subject of the marketing authorization application, which is sent for final approval to the CHMP. The CHMP recommendation is then sent to the European Commission, which adopts a decision binding in all EEA Member States. The maximum timeframe for the evaluation of a marketing authorization application for an ATMP is 210 days from receipt of a valid application, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CAT and/or 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 European Commission, who make the final decision to grant a marketing authorization, which is issued within 67 days of receipt of the EMA's recommendation. Accelerated assessment may be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts such a request, the timeframe of 210 days for assessment will be reduced to 150 days (excluding clock stops), but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that the application is no longer appropriate to conduct an accelerated assessment. The development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant EU guidelines, and the EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.

National marketing authorizations are 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 marketing authorization can be recognized in another Member States through the mutual recognition procedure. If the product has not received a national marketing authorization in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which an authorization is sought, one of which is selected by the applicant as the Reference Member State ("RMS"). If the RMS proposes to authorize the product, and the other Member States do not raise objections, the product is granted a national marketing authorization in all the Member States where the authorization was sought. Harmonization throughout all concerned Member States is achieved through procedures set forth in Regulation (EC) 1234/2008, including in cases of differences on the assessment between the relevant authorities of Member States.

Under the above-described procedures, before granting the MAA, the EMA or the competent authorities of the Member States of the EU make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety, and efficacy.

Now that the UK (which comprises Great Britain and Northern Ireland) has left the EU, Great Britain will no longer be covered by centralized marketing authorizations (under the Northern Ireland Protocol, centralized marketing authorizations will continue to be recognized in Northern Ireland). All medicinal products with a current centralized marketing authorization were automatically converted to Great Britain marketing authorizations on January, 1 2021. For products for which a centralized marketing authorization was granted in the EU prior to the end of 2023 (positive opinion of CHMP sufficient) and an application was received by the MHRA, the UK medicines regulator, prior to January 1, 2024, the MHRA may rely on a decision taken by the European Commission on the approval of a new marketing authorization in the centralized procedure, in order to more quickly grant a new Great Britain marketing authorization. For products for which a marketing authorization has been granted after January 1, 2024 (including centralised/decentralized marketing authorizations obtained in the EU or its Member States), companies may apply for a UK marketing authorization either under purely national rules or under the new UK International Recognition procedure ("IRP"). Under the IRP, applicants may benefit from a positive ruling of other "Reference Regulators", including EMA, Member States authorities, FDA and TGA and others. Under the IRP, applicants may also benefit from a fast track recognition, which allows for the granting of a UK marketing authorization within 60 days after validation of the submission by the MHRA if the applicable preconditions for this fast track ("Recognition A") are met. However, IRPs which include Great Britain orphan drug designation applications will not be eligible for the Recognition A fast track route.

Regulatory exclusivity

In the EU, innovative products authorized for marketing (i.e., reference products) may qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The data exclusivity period prevents generic or biosimilar applicants from relying on the preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization in the EU during a period of eight years from the date on which the reference product was first authorized in the EU. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until ten years have elapsed from the initial authorization of the reference product. The ten-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if an innovative medicinal product gains the prescribed period of data exclusivity, however, another company may market another version of the product if such company obtained marketing authorization based on a marketing authorization application with a completely independent data package of pharmaceutical tests, preclinical tests, and clinical trials.

Orphan designation and exclusivity

The criteria for designating an orphan medicinal product in the EU, are similar in principle to those in the U.S. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if the following criteria are fulfilled: (i) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in its development; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the marketing authorization application if the orphan designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Otherwise, orphan medicine marketing exclusivity may be revoked only in very select cases, such as if:

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

The aforementioned EU rules are generally applicable in the EEA.

PRIME designation

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines (PRIME) scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation, where the marketing authorization application will be made through the centralized procedure. Eligible products must target conditions for which where is an unmet medical need, i.e., there is no satisfactory method of diagnosis, prevention or treatment in the EU or, if there is, the new medicine will bring a major therapeutic advantage, and they must show potential to benefit patients with unmet medical needs based on early clinical data. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, a dedicated contact and rapporteur from the EMA's CHMP or Committee for Advanced Therapies are appointed early in PRIME scheme facilitating increased understanding of the product at the EMA's committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies. Where, during the course of development, a medicine no longer meets the eligibility criteria, support under the PRIME scheme may be withdrawn.

Brexit and the Regulatory Framework in the United Kingdom

In June 2016, the electorate in the UK voted in favor of leaving the EU (commonly referred to as "Brexit"), and the UK formally left the EU on January 31, 2020. There was a transition period during which EU pharmaceutical laws continued to apply to the UK, which expired on December 31, 2020. Initially, the EU and the UK concluded a trade and cooperation agreement, or TCA, which was provisionally applicable since January 1, 2021 and has been formally applicable since May 1, 2021. The TCA includes specific provisions concerning pharmaceuticals, which include the mutual recognition of GMP, inspections of manufacturing facilities for medicinal products and GMP documents issued but does not foresee wholesale mutual recognition of UK and EU pharmaceutical regulations. At present, Great Britain has moved to implement step-by-step legislation on the marketing, promotion and sale of medicinal products (under the Northern Ireland Protocol, the EU regulatory framework will continue to apply in Northern Ireland), application for marketing authorizations and the application for clinical trials. Therefore, while the regulatory regime in Great Britain may in part still align with EU regulations, the UK has now implemented new regulations and administrative processes for pharmaceutical processes, including the process for obtaining marketing authorizations in the UK (through a national marketing authorization or an IRP as described above) and, for clinical trials through the application of the UK Clinical Trial Regulation and the implementation of the combined review process by MHRA and HRA.

Human Capital

As of December 31, 2023, we had 268 full-time employees, of whom 256 were located in the U.S., eight in Spain, one in Switzerland, two in the UK and one in Sweden. Of these employees, 211 were primarily engaged in research and development activities and 57 were primarily engaged in general and administrative activities. We also engage the services of independent contractors and consultants as needed for special or temporary projects or specific expertise. None of our employees are represented by a labor union or covered by a collective bargaining agreement.

Compensation and Benefits Programs

Our human capital resources objectives include, as applicable, identifying, attracting, recruiting, retaining, incentivizing, developing, and integrating our existing and new employees, advisors, and consultants. The principal purposes of our equity and cash incentive plans are to attract, retain and reward personnel through the granting of stock-based and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives. We provide employee wages and benefits that are competitive within our industry, and we engage a nationally recognized outside compensation and benefits consulting firm to independently evaluate the effectiveness of our compensation and benefit programs and to provide benchmarking against our peers within the industry.

Diversity, Equity & Inclusion

We believe that developing a diverse, equitable and inclusive culture is critical to continuing to attract and retain the top talent necessary to deliver on our growth strategy. As such, we are investing in creating and maintaining a diverse, inclusive and safe work environment where our employees can feel inspired to deliver their workplace best every day. We regularly assess our benefit programs, employee engagement and turnover, recruitment initiatives, workforce diversity and other matters relevant to human capital management, and review those results with our board of directors on a periodic basis. All employees are responsible for upholding the Rocket Behaviors and the Rocket Code of Conduct, which form the foundation of our policies and practices.

Employee Development and Training

The development, recruitment and retention of our employees is a critical success factor for our company. To provide a meaningful experience for our employees, we offer training and development programs to increase our organizational learning and support the promotion and career development of our current employees.

Corporate Information

We were incorporated in Delaware in 1999 as Inotek Pharmaceuticals Corporation ("Inotek"). In January 2018, Inotek merged with Rocket Pharmaceuticals, Ltd. and changed its name to Rocket Pharmaceuticals, Inc. Our principal executive offices are located at 9 Cedarbrook Drive, Cranbury, NJ 08512, and our telephone number is (609) 659-8001. Our internet address is www.rocketpharma.com. We use our website as means of disclosing material non-public information and for complying with our disclosure obligations under Regulation FD. We make available on our website, free of charge, our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Our SEC reports can be accessed through the Investors section of our website. The SEC maintains a website that contains reports, proxy and information statements and other information regarding our filings at www.sec.gov. The information found on our website is not incorporated by reference into this report or any other report we file with or furnish to the SEC. Our common stock is listed on the NASDAQ Global Market under the symbol "RCKT."

Item 1A. Risk Factors

We operate in an industry that involves numerous risks and uncertainties. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Annual Report, including our financial statements and related notes hereto. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. The risks and uncertainties described below may change over time and other risks and uncertainties, including those that we do not currently consider material, may impair our business. In these circumstances, the market price of our common stock could decline.

Risks Related to Our Financial Condition and Capital Needs

Risks Related to Our Financial Condition and Operating History

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development efforts, we could be forced to delay, reduce, or eliminate our product development programs or commercial development efforts.

We are a late-stage gene therapy company with a limited operating history on which to base your investment decision. Gene therapy product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring, and developing product and technology rights, building out our R&D and manufacturing capabilities, and conducting preclinical and clinical R&D activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates and have funded our operations to date through proceeds from sales of our stock.

We have incurred net losses since our inception. We incurred net losses of $245.6 million, $221.9 million and $169.1 million for the years ended December 31, 2023, 2022 and 2021, respectively. As of December 31, 2023, we had an accumulated deficit of $959.4 million. Substantially all our operating losses have resulted from costs incurred in connection with our R&D programs, buildout of our manufacturing capabilities and from general and administrative ("G&A") costs associated with our operations. We expect to continue to incur significant expenses and operating losses for the foreseeable future as we intend to continue to conduct R&D, clinical trials, regulatory compliance activities, and internal and external manufacturing activities. If any of our product candidates are approved, sales and marketing activities, together with anticipated G&A expenses, would likely result in us continuing to incur significant losses for the foreseeable future.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates, we may not be successful in commercializing those product candidates if and when they are approved.

We have limited sales or marketing infrastructure and have no Company experience in the sale, marketing, or distribution of pharmaceutical products. To achieve commercial success for any approved candidate for which we retain sales and marketing responsibilities, we must either continue to develop our sales and marketing organization or outsource these functions to third parties. In the future, we may choose to continue to build a focused sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we establish marketing and other commercialization 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 commercialization personnel.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our product revenues or the profitability of these product revenues to us may be lower than if we were to market and sell any medicines we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our medicines effectively.

The amount of and our ability to use net operating losses and research and development credits to offset future taxable income may be subject to certain limitations and uncertainty.

Federal net operating losses generated in taxable years beginning after December 31, 2017 generally may not be carried back to prior taxable years, and while such federal net operating losses generated in taxable years beginning after December 31, 2017 will not be subject to expiration, the deduction for such net operating loss in any taxable year will be limited to 80% of our taxable income in such year, where taxable income is determined without regard to the net operating loss deduction itself. However, the Coronavirus Aid, Relief and Economic Security Act (the "CARES Act") repeals the 80% limitation on the utilization of such federal net operating losses for taxable years beginning after December 31, 2017 and beginning before January 1, 2021 and allows for federal net operating losses generated in taxable years beginning after December 31, 2017 and before January 1, 2021 to be carried back to each of the five taxable years preceding the taxable year in which the loss arises. This change in law temporarily allowing for the carryback of federal net operating losses is not expected to produce any material benefit for the issuer. As described above, we have incurred significant net losses since our inception and anticipate that we will continue to incur losses for the foreseeable future; and therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our net operating loss or tax credit carryforwards. Additionally, new tax laws could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, enacted many significant changes to the U.S. tax laws, including changes in corporate tax rates, which collectively may impact the utilization of our NOLs and other deferred tax assets, the deductibility of expenses, and the taxation of foreign earnings. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act, or any newly enacted federal tax legislation. The impact of changes under the Tax Act, the CARES Act, or future reform legislation could limit our ability to utilize our NOLs or increase our future U.S. tax expense and could have a material adverse impact on our business and financial condition.

In general, under Sections 382 and 383 of the Internal Revenue Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses, or net operating losses or tax credits, or credits, (including federal research and development tax credits) to offset future taxable income or taxes. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who owns at least 5% of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a specified testing period. As described below, we have experienced numerous "ownership changes" within the meaning of Section 382 of the Internal Revenue Code. Future changes in our stock ownership, many of which are outside of our control, could result in one or more additional ownership changes under Sections 382 and 383 of the Internal Revenue Code and further limit our ability to utilize our net operating losses and credits. Our net operating losses or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our net operating losses or credits if we undergo an ownership change prior to the utilization of all such net operating losses or credits.

Risks Related to Capital Needs

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

We expect to require substantial future capital in order to expand our gene therapy platforms, advance preclinical and clinical development for our current product candidates and other future product candidates, if any, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical and clinical activities. Also, if we obtain marketing approval for any of our current or future product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing, and distribution. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, limit or terminate our product development efforts or other operations. Furthermore, to the extent we raise additional funds through further issuances of equity or convertible debt securities, our existing stockholders could suffer significant dilution, and any new equity securities we issue could have rights, preferences, and privileges superior to those of holders of our common stock. Any debt financing secured by us in the future could involve restrictive covenants relating to our capital-raising activities and other financial and operational matters, which may make it more difficult for us to obtain additional capital and to pursue business opportunities, including potential acquisitions. Additionally, recent volatility in capital markets, rising interest rates and lower market prices for securities generally may affect our ability to access new capital on terms favorable to us, which may harm our liquidity, limit our ability to grow our business, pursue acquisitions or improve our operating infrastructure and restrict our ability to compete in our markets.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2023, our cash, cash equivalents and investments were $407.5 million. Our future capital requirements will depend on numerous factors, many of which are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

We have never generated any revenue from product sales and may never be profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory, pricing and reimbursement approvals necessary to commercialize our product candidates. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we successfully complete clinical studies;
- developing a sustainable, commercial-scale, reproducible, and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support preclinical and clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales force, marketing and distribution infrastructure;
- obtaining and maintaining a favorable market protection for our products, e.g., obtaining (and maintaining) orphan designation with market exclusivity in the EU, which in turn may depend on activities of third parties and other factors on which we have no influence;
- obtaining sufficient pricing and reimbursement for our product candidates from private and governmental payors;
- obtaining market acceptance of our product candidates and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- identifying and validating new gene therapy product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; and
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how.

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

Risks Related to Clinical Development and Product Regulatory Matters

Risks Related to Clinical Development of our Product Candidates

We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.

Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates. Clinical trials are expensive, time-consuming, and outcomes are uncertain.

Our experience with clinical trials has been limited. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial may be delayed or halted at any stage of testing for various reasons, including:

- failure of patients to enroll in the studies at the rate we expect;
- ineffectiveness of our product candidates;
- patients experiencing unexpected side effects or other safety concerns being raised during treatment;

- changes in governmental regulations or administrative actions;
- failure to conduct studies in accordance with required clinical practices;
- inspection of clinical study operations or study sites by the FDA, the EMA or other regulatory authorities, resulting in a clinical hold;
- insufficient financial resources;
- insufficient supplies of drug product to treat patients in our ongoing and planned clinical trials;
- political unrest or natural disasters at domestic or foreign clinical sites;
- a shutdown of the U.S. government, including the FDA;
- public health crises such as pandemics and epidemics.

In addition, to the extent we seek to obtain regulatory approval for our product candidates in foreign countries, our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with Contract Research Organizations ("CROs") and physicians;
- absence in some countries of established groups with sufficient regulatory expertise for review of LV and AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate planned clinical trials, the occurrence of any of which would harm our business, financial condition, results of operations and prospects.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. We may not be able to identify, recruit and enroll enough patients, or those with required or desired characteristics, to complete clinical trials in a timely manner. Patient enrollment and trial completion is affected by numerous factors including:

- severity of the disease under investigation and size of the patient population;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions for which we plan to evaluate our current product candidates are rare genetic diseases with limited patient pools from which to draw for clinical studies. The process of identifying and diagnosing patients may prove costly. In some cases, potential patients may be located outside of the U.S., and immigration related issues, including government policy changes, may introduce additional delays into the enrollment process. Finally, the treatment process for our LV programs requires that the cells be obtained from patients and then shipped to a transduction facility within the required timelines, and this may introduce unacceptable shipping-related delays to the process.

Preliminary, interim or topline results in our ongoing clinical studies may not be indicative of results obtained when these studies are completed. Furthermore, success in early clinical studies may not be indicative of results obtained in later studies.

We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule. Study designs and results from previous or ongoing studies and clinical trials are not necessarily predictive of future study or clinical trial results, and initial or interim results may not continue or be confirmed upon completion of the study or trial. Furthermore, our product candidates may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies. We cannot guarantee that any of these studies will ultimately be successful or that preclinical or early-stage clinical studies will support further clinical advancement or regulatory approval of our product candidates.

From time to time, we may publicly disclose interim or topline data from our preclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data are available. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercial viability of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

Our product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

Gene therapy is still a relatively new approach to disease treatment and adverse side effects could develop with our product candidates.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction soon after administration which could substantially limit the effectiveness and durability of the treatment. If certain side effects are observed in testing of our potential product candidates, we may decide or be required to halt or delay further clinical development of our product candidates. The FDA or other regulatory authorities may require us to halt or delay clinical development of our product candidates for reasons unrelated to new drug-related safety events being observed. For example, our Phase 1 clinical trial of RP-A501 for the treatment of DD was placed on clinical hold by the FDA in May of 2021 following a thrombotic microangiopathy event believed to be due to immune-mediated complement activation. We modified the study protocol and other supporting documents with revised guidelines for patient selection and safety management and the clinical hold was lifted in August 2021.

In addition to side effects caused by the product candidate, the administration process or related procedures associated with a given product candidate also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. Under certain circumstances, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Moreover, if we elect or are required, to not initiate or to delay, suspend or terminate any ongoing or future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated.

Furthermore, if undesirable side effects caused by our product candidate are identified following regulatory approval of a product candidate, such as in long-term follow-up studies, several potentially significant negative consequences could result, including reputational harm and regulatory authorities suspending or withdrawing approvals of such product candidate, requiring additional warnings on the label or requiring that we change the way a product candidate is administered or that we conduct additional clinical trials.

Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Risks Related to Government Regulation

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

We have concentrated our R&D efforts to date on a gene therapy platform, and our future success depends on the successful development of viable gene therapy product candidates.

The clinical study requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, relatively few gene and cell therapy products have received marketing authorization in the U.S. or the EU, including Novartis Pharmaceuticals' Kymriah and Zolgensma (developed by AveXis), Kite Pharma's Yescarta, GlaxoSmithKline's Strimvelis, Spark Therapeutics' Luxturna, Vertex Pharmaceuticals' Casgevy and Bluebird Bio's Lyfgenia. It is therefore difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA may not be indicative of what the FDA may require for approval. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approvals necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue and our business, financial condition, results of operations and prospects could be materially harmed.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, FDA's CBER may require us to perform additional nonclinical studies or clinical trials that may increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our gene therapy product candidates or lead to significant post-approval limitations or restrictions. Additionally, the FDA continues to develop its approach to assessing gene and cell therapy products. In January 2020, FDA released its final guidance with recommendations for long-term follow-up studies of patients following human gene therapy administration due to the increased risk of undesirable and unpredictable outcomes with gene therapies that may present as delayed adverse events. The final guidance advises that patients treated with gene therapies that incorporate integrating vectors, such as LVs, undergo long-term safety and efficacy follow up of fifteen years post therapy while patients treated with gene therapies that incorporate AAV vectors undergo long-term safety and efficacy follow-up as long as five years post therapy. We cannot be certain whether such guidance, or others that FDA may issue, will adversely impact our gene therapy candidates or the duration or expense of any applicable regulatory development and review processes.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the U.S., certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of institutional biosafety committees, or IBCs, as set forth in the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Under the NIH Guidelines, recombinant and synthetic nucleic acids are defined as: (i) molecules that are constructed by joining nucleic acid molecules and that can replicate in a living cell (i.e., recombinant nucleic acids); (ii) nucleic acid molecules that are chemically or by other means synthesized or amplified, including those that are chemically or otherwise modified but can base pair with naturally occurring nucleic acid molecules (i.e., synthetic nucleic acids); or (iii) molecules that result from the replication of those described in (i) or (ii). Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

In addition, the EMA's Committee for Advanced Therapies ("CAT") and other regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate product revenue, and our business, financial condition, results of operations and prospects would be materially harmed.

Even though we have obtained orphan designation for certain of our product candidates, we may not be able to realize the benefits of such designation, including potential marketing exclusivity of our product candidates, if approved.

Regulatory authorities in some jurisdictions, including the U.S., EU and other major markets, may designate drugs intended to treat conditions or diseases affecting relatively small patient populations as orphan drugs. The FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the U.S., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, the European Commission, based on the recommendation of the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition and either (i) such condition affects not more than 5 in 10,000 persons in the EU; or (ii) without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug or biologic product. In either case, the applicant for orphan designation must also demonstrate that 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 must be of significant benefit compared to products available for the condition.

We have received orphan designation from the FDA and the European Commission for RP-L102 for the treatment of FA, for RP-L201 for the treatment of LAD-I, for RP-L301 for the treatment of PKD, and FDA orphan drug designation for RP-A501 for treatment of DD and RP-A601 for the treatment of PKP2-ACM. To date, we have not requested orphan drug designation (or the foreign equivalent) for any other product candidates, and even if we do in the future there can be no assurances that the FDA or foreign regulatory authorities will grant any of our product candidates such designation. Additionally, the designation of any of our product candidates as an orphan product does not mean that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or foreign regulatory authorities from approving another marketing application for a product that constitutes the same drug (or "similar medicinal product" in the EEA, which 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) treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), or if in the EU a "similar medicinal product" is approved before we obtain a market authorizations for our product, we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the U.S. and 10 years in the EU. The exclusivity period in the EU may be extended by an additional two years if the applicant enjoys the incentives and rewards granted for including the results of additional pediatric studies in its product information. On the other hand, the exclusivity period in the EU can be reduced to six years if a product no longer meets the criteria for orphan drug designation, such designation is revoked by the sponsor or expires, including if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition in the U.S. Even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Additionally, the U.S. federal courts may interpret the orphan drug statutory or regulatory provisions in way that reduces or eliminates any exclusivity that may attach to our product candidates.The FDA may further reevaluate its regulations and policies related to orphan designation and orphan drug exclusivity. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

In the EU, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

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

A Fast Track or regenerative medicine advanced therapy, or RMAT, designation by the FDA, or a PRIority MEdicines, or PRIME, designation by the EMA, even if granted for any of our current or future product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our current product candidate and any future product candidates will receive marketing approval.

If a product candidate is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for FDA Fast Track designation for a particular indication. We have received Fast Track designation for RP-A501 for DD, RP-L102 for FA, RP-L201 for LAD-I and RP-L301 for PKD. We may seek Fast Track designation for future product candidates, but there is no assurance that the FDA will grant this status to any of our proposed product candidates.

A company may request RMAT designation of its product candidate, and FDA may grant such designation if the product meets the following criteria: (i) it is a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (ii) it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (iii) preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. We have received RMAT designation for RP-A501 for DD, RP-L102 for FA, RP-L201 for LAD-I and RP-L301 for PKD. RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and potential eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion of trials to additional sites.

PRIME designation is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need. To qualify for PRIME designation, product candidates require early clinical evidence that the therapy has the potential to offer a therapeutic advantage over existing treatments or benefits patients without treatment options. We have received PRIME designation for RP-L102 for FA, RP-L201 for LAD-I, RP-L301 for PKD and RP-A501 for DD. Among the benefits of PRIME are the appointment of a rapporteur to provide continuous support and help build knowledge ahead of a marketing authorization application, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review earlier in the application process.

The FDA has broad discretion whether or not to grant Fast Track or RMAT designation, and the EMA has broad discretion whether or not to grant PRIME designation, so even if we believe a particular product candidate is eligible for such designations, there can be no assurance that the FDA or EMA would decide to grant it. Even if we do receive Fast Track, RMAT or PRIME designation, we may not experience a faster development process, review or approval compared to conventional development, review, and approval timelines, and receiving a Fast Track, RMAT or PRIME designation does not change the standards for the product approval. In addition, the FDA may withdraw Fast Track or RMAT designation and the EMA may revoke PRIME designation if it believes that the designation is no longer supported by data from our clinical development program.

Accelerated approval by the FDA, and conditional approval by the EMA, may not lead to a faster development process or regulatory review and does not increase the likelihood that our product candidates will receive marketing approval. If we are not successful with this process, the development or commercialization of our product candidates for which we seek accelerated approval or conditional approval could be delayed, abandoned or become significantly more costly.

We may seek approval of our product candidates using the FDA's accelerated approval and the EMA's conditional approval pathways. While we may utilize trial designs to support accelerated approval, such product candidates may not be subject to faster development or regulatory review timelines.

A product may be eligible for accelerated approval by the FDA if it treats a serious or life-threatening condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. As a condition of accelerated approval, the FDA may impose specific obligations with defined timelines, including to perform adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence. In addition, the FDA currently requires, unless otherwise informed by the agency, pre-approval of promotional materials for products receiving accelerated approval, which could adversely impact the timing of the commercial launch of a product. If the FDA or the EMA do not approve our product candidates for which we seek accelerated approval or conditional approval, but instead require the completion of a full Phase 3 clinical trial or trials prior to the filing of marketing applications, the development and commercialization timeline of such product candidates will be delayed. Even if we do receive accelerated approval or conditional approval, we may not ultimately receive full approval from the regulatory agencies. The additional data generated through post-marketing clinical trials may not confirm that the benefit-risk balance of any of our product candidates that receive accelerated approval is positive or the burden to further complete the obligations may become too high. Additionally, the Consolidated Appropriations Act of 2023, enacted on December 29, 2022, contained revisions to the accelerated approval process that provide FDA with additional authority to enforce the post-market study requirements and withdraw approvals more rapidly when holders of accelerated approvals fail to comply with post-approval clinical study requirements.

In the EU, the conditional marketing authorization is subject to an annual renewal procedure that assesses the marketing authorization holder's compliance with the specific obligations of the authorization. If conditions are not complied with, the EMA may decide to extend the timeline for the existing obligations, change the scope of such obligations or add new obligations, which may require additional financial resources and time. We may not be able to comply with such changes or additional obligations and may need to withdraw the marketing authorization. The EMA may also decide not to renew the conditional marketing authorization, although such measure is rarely applied in practice. An analysis of reimbursement decisions for conditionally authorized medicines in the EU has shown some delays in the timeline for reaching a positive health technology recommendation. If this happens for any product candidate for which we seek conditional approval, it may delay the timing and success of the commercialization of such product. Finally, if new data obtained from fulfilment of the conditions of the conditional authorization or otherwise show that our product's benefits no longer outweigh its risks, the EMA can take regulatory action, such as suspending or revoking the conditional marketing authorization.

We have received rare pediatric disease designation for RP-A501 for DD, RP-L102 for FA, and RP-L201 for LAD-I. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher.

We have received rare pediatric disease designation for RP-A501 for DD, RP-L102 for FA, and RP-L201 for LAD-I. Designation of a biological product as a product for a rare pediatric disease does not guarantee that a BLA for such biological product will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the Federal Food, Drug, and Cosmetic Act ("FDCA"), we will need to request a rare pediatric disease priority review voucher in our original BLA for our product candidates for which we have received rare pediatric disease designation. The FDA may determine that a BLA for any such product candidates, if approved, does not meet the eligibility criteria for a priority review voucher.

The authority for the FDA to award rare pediatric disease priority review vouchers for biological products after September 30, 2024 is currently limited to biological products that receive rare pediatric disease designation on or prior to September 30, 2024, and FDA may only award rare pediatric disease priority review vouchers through September 30, 2026. However, it is possible the authority for FDA to award rare pediatric disease priority review vouchers will be further extended by Congress.

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

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. We have not received approval from regulatory authorities in any jurisdiction to market any of our product candidates. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, issue a complete response letter, or ultimately, we may not be able to obtain regulatory approval. In addition, we may experience delays or rejections if an FDA Advisory Committee recommends disapproval or restrictions on use. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative actions, or changes in regulatory authority policy during the period of product development, clinical trials and the review process. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of data obtained from preclinical and clinical testing could delay, limit or prevent the receipt of marketing approval for a product candidate.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or other labeling changes. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. Regulatory authorities may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a Risk Evaluation and Mitigation Strategy, or REMS, or equivalent requirement. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects.

We may never obtain FDA or EMA approval for any of our product candidates in the U.S. or the EU, and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize our full market potential.

In order to eventually market any of our product candidates in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements regarding safety and efficacy on a jurisdiction-by-jurisdiction basis. Approval by the FDA in the U.S. or the EMA in the EU, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, preclinical studies and clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. 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. While the foreign regulatory approval process involves similar risks to those associated with FDA or EMA approval, regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory obligations and continued regulatory scrutiny.

Even if we obtain regulatory approval in a jurisdiction, we will remain subject to ongoing regulatory obligations and continued regulatory scrutiny. The applicable regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions once a product candidate is approved. Additionally, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA and must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP and cGTP, as well as adherence to commitments made in the BLA. For certain commercial prescription biological products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the U.S. If we or a regulatory agency discover previously unknown problems with a product, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product or the manufacturing facility. Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may take a variety of actions, including:

- issuing a warning letter asserting that we are in violation of the law;
- seeking an injunction or impose civil or criminal penalties or monetary fines;
- suspending any ongoing clinical studies;
- refusing to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;
- seizing products; or
- refusing to allow us to enter into supply contracts, including government contracts.

In addition, the FDA's policies, and those of comparable foreign regulatory authorities, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative actions, either in the U.S. or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval which we may have obtained and we may not achieve or sustain profitability, which would materially harm our business, financial condition, results of operations and prospects.

If approved, our product candidates may face competition from biosimilars approved through an abbreviated regulatory pathway.

The ACA includes a subtitle called the BPCIA, which created an abbreviated approval pathway for biologic products that are biosimilar to or interchangeable with an FDA-licensed reference biologic product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. The law is complex and, as a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

We believe that any of our product candidates approved as a biologic product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to Congressional action or otherwise, or that the FDA will not consider our investigational medicines to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biologic products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

The U.S. and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates or any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. See the section entitled, "*Business — Government Regulation — Healthcare Legislative Reform*".

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. Federal Government will pay for healthcare drugs and services, which could result in reduced demand for our drug candidates or additional pricing pressures.

Individual states in the U.S. have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain drug access and marketing cost disclosure and transparency measures, and designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, financial condition, results of operations and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our drugs or put pressure on our drug pricing, which could negatively affect our business, financial condition, results of operations and prospects.

The United Kingdom's withdrawal from the EU, or Brexit, could result in increased regulatory and legal complexity, which may make it more difficult for us to do business in Europe and impose additional challenges in securing regulatory approval of our product candidates in Europe and/or the United Kingdom.

We currently have clinical trial sites in the United Kingdom, contract laboratories in the United Kingdom conducting testing for our global clinical trials, and other collaborators and potential collaborators in the United Kingdom and throughout Europe. Pursuant to Article 50 of the Treaty on EU, the UK ceased being a Member State of the EU on January 31, 2020.

There was a transition period during which EU pharmaceutical laws continued to apply to the UK, which expired on December 31, 2020. Initially, the EU and the UK concluded a trade and cooperation agreement, or TCA, which was provisionally applicable since January 1, 2021 and has been formally applicable since May 1, 2021. The TCA includes specific provisions concerning pharmaceuticals, which include the mutual recognition of GMP, inspections of manufacturing facilities for medicinal products and GMP documents issued but does not foresee wholesale mutual recognition of UK and EU pharmaceutical regulations. At present, Great Britain has moved to implement step-by-step legislation on the marketing, promotion and sale of medicinal products (under the Northern Ireland Protocol, the EU regulatory framework will continue to apply in Northern Ireland), application for marketing authorizations and the application for clinical trials. Therefore, while the regulatory regime in Great Britain may in part still align with EU regulations, the UK has now implemented new regulations and administrative processes for pharmaceutical processes, including the process for obtaining marketing authorizations in the UK (through a national marketing authorization or an IRP as described above) and, for clinical trials through the application of the UK Clinical Trial Regulation and the implementation of the combined review process by MHRA and HRA.

The cumulative effects of the disruption to the regulatory framework may add considerably to the development lead time to marketing authorization and commercialization of products in the EU and/or the United Kingdom. It is possible that there will be increased regulatory complexities which can disrupt the timing of our clinical trials and regulatory approvals. In addition, changes in, and legal uncertainty with regard to, national and international laws and regulations may present difficulties for our clinical and regulatory strategy.

Risks Related to Noncompliance with Applicable Laws or Regulations

If we are successful in commercializing any product, our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain regulatory approval. Our arrangements with third-party payors, healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any products for which we obtain regulatory approval. See the section entitled, "*Business — Government Regulation — Anti-Kickback and False Claims Laws and Other Regulatory Matters*."

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Efforts to ensure that our business arrangements with third parties, and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant administrative, civil and criminal penalties, damages, fines, disgorgement, the exclusion from participation in federal and state healthcare programs, individual imprisonment, reputational harm, and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, any of which could adversely affect our ability to operate our business and our results of operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other healthcare providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

We are subject to stringent laws, rules, regulations, policies, industry standards and contractual obligations regarding data privacy and security and may be subject to additional related laws and regulations in jurisdictions into which we expand. Many of these laws and regulations are subject to change and reinterpretation and could result in claims, changes to our business practices, monetary penalties, increased cost of operations or other harm to our business.

The regulatory framework for privacy and personal information security issues worldwide is evolving rapidly and likely to remain uncertain for the foreseeable future. The U.S. federal and various state, local and foreign government bodies and agencies have adopted or are considering adopting laws, rules, regulations and standards regarding, the collection, distribution, use, disclosure, storage, security and other processing of personal information. For example, HIPAA imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. We may obtain health information from third parties, including research institutions from which we obtain clinical trial data, that are subject to privacy and security requirements under HIPAA and its implementing rules and regulations. Depending on the facts and circumstances, we could be subject to significant penalties if we obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Outside of the U.S., relevant legal requirements continue to evolve. For example, the collection and use of health data and other personal data including data collected in clinical trials is governed in the EU by the General Data Protection Regulation ("GDPR"), which imposes substantial obligations upon companies and new rights for individuals. The GDPR also forms part of the law of Great Britain (England and Wales, Scotland and Northern Ireland) by virtue of section 3 of the European Union (Withdrawal) Act 2018 and as amended by the Data Protection, Privacy and Electronic Communications (Amendments etc.) (EU Exit) Regulations 2019 (SI 2019/419) ("UK GDPR"). Failure to comply with the GDPR may result in fines of the higher of (i) €20,000,000 or (ii) 4% of the preceding fiscal year's total annual global revenues of the noncompliant company, among other administrative penalties. The GDPR has increased our responsibility and liability in relation to personal data that we may process, and we may be required to implement additional measures in an effort to comply with the GDPR and with other laws, rules, regulations and standards in the EU and United Kingdom relating to privacy and data protection. This may be onerous and if our efforts to comply with GDPR or other applicable laws, rules, regulations and standards are not successful, or are perceived to be unsuccessful, it could adversely affect our business. Further, following the July 2020 Court of Justice of the EU ("CJEU") decision invalidating the EU-U.S. Privacy Shield, there remains uncertainty regarding the appropriate mechanism for transferring personal data to the U.S. The CJEU's decision and other regulatory guidance or developments may impose additional obligations with respect to the transfer of personal data from the EU to the U.S., all of which could restrict our activities in those jurisdictions, limit our ability to provide our products and services in those jurisdictions, require us to modify our policies and practices, and to engage in additional contractual negotiations, or increase our costs and obligations and impose limitations upon our ability to efficiently transfer personal data from the EU to the U.S.

In the U.S., a variety of data privacy, protection and security laws, rules, regulations and standards potentially may apply to our activities, such as state data breach notification laws, state personal data privacy laws (for example, the California Consumer Privacy Act of 2018 as amended by the California Privacy Rights Act effective January 1, 2023 ("CCPA")), state health information privacy laws, and federal and state consumer protection laws. The CCPA requires covered businesses that process personal information of California residents to disclose their data collection, use, sharing and retention practices, provides California residents with data privacy rights (including the ability to opt out of certain disclosures of personal information including for certain advertising purposes), imposes operational requirements for covered businesses, provides for significant civil penalties for violations as well as a private right of action for certain data breaches and statutory damages (that is expected to increase data breach class action litigation and result in significant exposure to costly legal judgements and settlements). Although there are limited exemptions for clinical trial data under the CCPA, the CCPA and other similar laws could impact our business activities, depending on their interpretation. Other state legislatures have enacted or are currently contemplating, and may pass, their own comprehensive data privacy and security laws, with potentially greater penalties and more rigorous compliance requirements, and laws in all 50 states require businesses to provide notice to customers whose personal data has been disclosed as a result of a data breach. Finally, federal, state and foreign laws, rules, regulations and standards may apply generally to the privacy and security of information we maintain, and may differ from each other significantly, thus complicating compliance efforts and potentially requiring us to undertake additional measures to comply with them.

With HIPAA, GDPR, CCPA, and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and making necessary changes to our policies and practices and may incur significant costs and expenses in an effort to do so.

We may make public statements about our use, collection, disclosure and other processing of personal data through our privacy policies, information provided on our website and press statements. Although we endeavor to comply with our public statements and documentation, we may at times fail to do so or be alleged to have failed to do so. Any failure or perceived failure by us or our vendors or service providers to comply with our applicable policies or notices relating to privacy or data protection, our contractual or other obligations to third parties, or any of our other legal obligations, laws, rules, regulations and standards relating to privacy or data protection, may result in governmental investigations or enforcement actions, litigation, claims and other proceedings, harm our reputation, and could result in significant liability.

We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.

Our operations, including our development, testing and manufacturing activities, are subject to numerous environmental, health and safety laws and regulations. These laws and regulations govern, among other things, the controlled use, handling, release and disposal of and the maintenance of a registry for, hazardous materials and biological materials, such as chemical solvents, human cells, carcinogenic compounds, mutagenic compounds and compounds that have a toxic effect on reproduction, laboratory procedures and exposure to blood-borne pathogens. If we fail to comply with such laws and regulations, we could be subject to fines or other sanctions.

As with other companies engaged in similar activities, we face a risk of environmental liability inherent in our activities, including liability relating to releases of or exposure to hazardous or biological materials. Environmental, health and safety laws and regulations are becoming more stringent. We may be required to incur substantial expenses in connection with future environmental compliance or remediation activities, in which case, the production efforts of our third-party manufacturers or our development efforts may be interrupted or delayed.

Risks Related to Manufacturing, Commercialization and Development of Our Product Candidates

Risks Related to Manufacturing our Product Candidates

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.

We currently have development, manufacturing, and testing agreements with third parties to manufacture supplies of certain of our product candidates. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, public health crises such as pandemics and epidemics, disruption in utility services, human error or disruptions in the operations of suppliers.

Our product candidates require processing steps that are more complex than those required for small molecule pharmaceuticals. The facilities used by our contract manufacturers to manufacture our product candidates must be inspected by the FDA pursuant to pre-approval inspections that will be conducted after we submit our marketing applications to the FDA. We do not control the manufacturing process of, and will be completely dependent on, our contract manufacturers for compliance with cGMPs in connection with the manufacture of certain of our product candidates. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to pass regulatory inspections and/or maintain regulatory compliance for their manufacturing facilities and we may need to find alternatives, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to attractive development programs. Problems in third-party manufacturing processes or facilities also could restrict our ability to complete our clinical trials in a timely manner or meet market demand for our products. Additionally, should our manufacturing agreements with third parties be terminated for any reason, there may be a limited number of manufacturers who would be suitable replacements and it could take a significant amount of time to transition the manufacturing to a replacement. In some cases, the technical skills required to manufacture our products or product candidates may be unique or proprietary to the original manufacturer 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. Changes to the manufacturing process or the transfer or setup of new manufacturing facilities could require that we conduct bridging studies before being able to proceed with either clinical or commercial manufacturing activities. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials. Further, the shift would likely be expensive and time-consuming, particularly since the new facility would need to comply with the necessary regulatory requirements or may require approval before selling any products manufactured at that facility.

We have limited experience in manufacturing, and there can be no assurance that we will be able to manufacture products at the scale our business may require.

We have historically relied on third parties to manufacture supplies of our product candidates. We have completed a build-out of a new manufacturing facility in Cranbury, New Jersey, and have recently completed two DD AAV cGMP production batches.

Although some of our employees have experience in the manufacturing of biopharmaceutical products from prior employment at other companies, we as a company have very limited prior experience in manufacturing. As a manufacturer of pharmaceutical products, we will be required to demonstrate and maintain compliance with cGMP requirements related to production processes, quality control and assurance and recordkeeping. Furthermore, establishing and maintaining manufacturing operations may require a reallocation of other resources, particularly the time and attention of certain of our senior management as well as potentially significant capital expenditures. Any failure or delay in the development of our manufacturing capabilities could adversely impact the development or commercialization of our product candidates.

Our manufacturing facilities are subject to significant government regulations and approvals, which are often costly and could result in adverse consequences to our business if we fail to comply with the regulations or maintain the approvals.

We must comply with cGMP requirements, as set out in statute, regulations and guidance. We may encounter difficulties in achieving quality control and quality assurance and may experience shortages in qualified personnel. We are subject to inspections by the FDA and comparable agencies in other jurisdictions to confirm compliance with applicable regulatory requirements. Any failure to follow cGMP or other regulatory requirements or delay, interruption or other issues that arise in the manufacture, fill-finish, packaging, or storage of our product candidates as a result of a failure of our facilities or the facilities or operations of third parties to comply with regulatory requirements or pass any regulatory authority inspection could significantly impair our ability to develop and commercialize our product candidates, including leading to significant delays in the availability of drug product for our clinical trials or the termination or hold on a clinical trial, or the delay or prevention of a filing or approval of marketing applications for our product candidates. Significant noncompliance could also result in the imposition of sanctions, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approvals for our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could damage our reputation. If we are not able to maintain regulatory compliance, we may not be permitted to market our product candidates and/or may be subject to product recalls, seizures, injunctions, or criminal prosecution.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit the potential commercialization of any products that we may develop.

We face inherent risks of product liability exposure related to the testing of our product candidates in human clinical trials and will face even greater product liability risks if we commercially sell any approved products. Product liability claims may be brought against us by subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities.

We currently hold product liability insurance coverage at a level that we believe is customary for similarly situated companies and adequate to provide us with insurance coverage for foreseeable risks, but which may not be adequate to cover all liabilities that we may incur. 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. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain regulatory approval for our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products that receive regulatory approval. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

Risks Related to Commercialization of our Product Candidates

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

Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be covered and paid by government authorities and other third-party payors, such as private health insurers and health maintenance organizations, which we cannot guarantee. We have not commenced efforts to have our product candidates reimbursed by government or third-party payors. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to successfully commercialize our products. If coverage is provided, but only at limited levels, the reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payor will pay for the product. Payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. A decision by a payor not to cover our product candidates could reduce physician utilization of our products once approved and have a material adverse effect on our sales, results of operations and financial condition. See the section entitled, "*Business — Government Regulation — Coverage and Reimbursement*."

In the U.S., the principal decisions about coverage and reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services ("CMS"), an agency within the U.S. Department of Health and Human Services ("HHS"), as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow the CMS to a substantial degree. It is difficult to predict what the CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Factors payors consider in determining reimbursement include whether the product is a covered benefit under its health plan, whether the product is safe, effective, and medically necessary, whether it is cost-effective and whether the product is experimental or investigational.

Third-party payors are increasingly limiting both coverage and the level of reimbursement of new drugs. They may also impose strict prior authorization requirements and/or refuse to provide any coverage of uses of approved products for medical indications other than those for which the FDA has granted market approvals. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly approved drugs. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price and best price. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important to successful commercialization of our product candidates.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. A Member State may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our product candidates.

Historically, products launched in the EU do not follow price structures of the U.S. and generally prices tend to be significantly lower. In some cases, the reimbursement price of one Member State may have impact on the pricing level in other Member States, which may result in an incentive not to market products in some markets to prevent price reductions or erosions in other markets.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

We are engaged in gene therapy for severe genetic and rare diseases, which is a competitive and rapidly changing field. Although we are not currently aware of any gene therapy competitors addressing any of the same indications as those in our pipeline, we may have competitors both in the U.S. and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions.

Our potential competitors may have substantially greater financial, technical and other resources, such as larger R&D staff, more robust manufacturing capabilities and more experienced marketing and manufacturing organizations. These competitors may succeed in developing, acquiring or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization and market penetration than us. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against those of our competitors.

In addition, if our patent rights were to expire or be successfully challenged, we could face increased litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize, thereby causing harm to our business, financial condition, results of operations and prospects.

The commercial success of any of our product candidates will depend upon the degree of market acceptance of gene therapy by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the U.S., the EMA in the EU and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically beneficial, cost-effective and safe. If any products that we commercialize do not achieve an adequate level of acceptance by physicians, patients, health care payors and others in the medical community, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in preclinical studies and clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments, including the prevalence and severity of any side effects;
- the cost of our treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the EMA;
- patient and physician awareness of, and willingness to seek, gene therapy;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is approved and launched and is subject to change over time if adverse long-term follow-up data become available after approval. The failure of any of our product candidates to achieve market acceptance could materially harm our business, financial condition, results of operations and prospects.

Ethical, legal, and social issues may reduce demand for any gene therapy products for which we obtain marketing approval.

Prior to receiving certain gene therapies, patients may be required to undergo genetic testing. Genetic testing has raised concerns regarding the appropriate utilization and the confidentiality of information provided by genetic testing. Genetic tests for assessing a person's likelihood of developing a chronic disease have focused public attention on the need to protect the privacy of genetic information. Concerns have been expressed that insurance carriers and employers may use these tests to discriminate on the basis of genetic information, resulting in barriers to the acceptance of genetic tests by consumers. This could lead to governmental authorities restricting genetic testing or calling for limits on or regulating the use of genetic testing, particularly for diseases for which there is no known cure. Any of these scenarios could decrease demand for any products for which we obtain marketing approval.

Risks Related to Development of our Pipeline and Research and Development Activities

We may not be successful in our efforts to expand our pipeline of additional product candidates for development.

Our business model is centered on applying our expertise in rare genetic diseases by establishing focused selection criteria to develop and advance a portfolio of gene therapy product candidates through development into commercialization. We may not be able to continue to identify and develop new product candidates in addition to the pipeline of product candidates that our efforts to date have resulted in. Even if we are successful in continuing to expand our pipeline, any potential product candidates that we identify may not be suitable for clinical development. If we do not successfully identify, develop and commercialize product candidates, we will not be able to obtain product revenue in future periods, which would likely result in significant harm to our financial position and results of operations.

The success of our R&D activities, clinical testing and commercialization, upon which we primarily focus, is uncertain.

Our primary focus is on our R&D activities and the clinical testing and commercialization of our product candidates, and we anticipate that we will remain principally engaged in these activities for an indeterminate, but substantial, period. R&D was our most significant operating expense for the year ended December 31, 2023. R&D activities, including the conduct of clinical studies, by their nature, preclude definitive statements as to the time required and costs involved in reaching certain objectives. Actual R&D costs, therefore, could significantly exceed budgeted amounts and estimated timeframes may require significant extension. Cost overruns, unanticipated regulatory delays or demands, unexpected adverse side effects or insufficient therapeutic efficacy will prevent or substantially slow our R&D effort and our business could ultimately suffer.

Risks Related to Third Parties

We rely on third parties to conduct certain aspects of our preclinical studies and clinical trials and perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business, financial condition and results of operations could be substantially harmed.

We have relied upon and plan to continue to rely upon third parties, including CROs, medical institutions, and contract laboratories for certain aspects of our ongoing preclinical and clinical programs. Nevertheless, we maintain responsibility for ensuring that each of our clinical trials and preclinical studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on these third parties does not relieve us of our regulatory responsibilities. We and our vendors are required to comply with the current requirements of GMP, good clinical practice ("GCP"), and good laboratory practice ("GLP"), which are a collection of laws and regulations enforced by the FDA, the EMA or comparable foreign authorities for our drug candidates in clinical development.

Regulatory authorities enforce these regulations through periodic inspections of preclinical study and clinical trial sponsors, principal investigators, preclinical study and clinical trial sites, and other contractors. If we or any of our vendors fail to comply with applicable regulations, the data generated in our preclinical studies and clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign authorities may require us to perform additional preclinical studies and clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced consistent with GMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the development and regulatory approval processes.

If any of our relationships with these third parties, medical institutions, clinical investigators or contract laboratories terminate, we may not be able to enter into arrangements with alternative CROs on commercially reasonable terms, or at all. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether they devote sufficient time and resources to our ongoing preclinical and clinical programs.

Switching or adding additional CROs, medical institutions, clinical investigators or contract laboratories involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work replacing a previous CRO. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, we cannot guarantee that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on our business, financial condition or results of operations.

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing our product candidates.

We may seek to establish strategic partnerships for developing and/or commercializing certain of our product candidates due to relatively high capital costs required to develop the product candidates, manufacturing constraints or other reasons. We may not be successful in our efforts to establish such strategic partnerships or other alternative arrangements for our product candidates for several reasons, including because our R&D pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate efficacy or market opportunity. In addition, we may be restricted under existing agreements from entering into future agreements with potential collaborators.

If we are unable to reach agreements with suitable licensees or collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, reduce or delay our development program, delay our potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to independently fund development or commercialization activities, we may need to obtain additional expertise and additional capital, which may not be available on acceptable terms or at all. If we fail to enter into collaboration arrangements and do not have sufficient funds or expertise to undertake necessary development and commercialization activities, we may not be able to further develop our product candidates and our business, financial condition, results of operations and prospects may be materially harmed.

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

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies 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 biologics or modifications to approved biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities.

Additionally as of May 26, 2021, the FDA noted it is continuing to ensure timely reviews of applications for medical products during the COVID-19 pandemic in line with its user fee performance goals; however, the FDA may not be able to continue its current pace and approval timelines could be extended, including where a pre-approval inspection or an inspection of clinical sites is required, due to new variants of the COVID-19 pandemic or any future pandemic. Should FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency could issue a complete response letter or defer action on the application until an inspection can be completed. During the COVID-19 public health emergency, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. Regulatory authorities outside the U.S. may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic or any future pandemic and may experience delays in their regulatory activities. If the FDA becomes unable to continue its current level of performance, we could experience delays and setbacks for our product candidates and for any approvals we may seek which could adversely affect our business.

Risks Related to Our Intellectual Property

Our rights to intellectual property for the development and commercialization of our product candidates are subject to the terms and conditions of licenses granted to us by others.

We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our gene therapy product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to license our platform or develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories not included in our licenses.

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

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering technology that we license from third parties. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be impacted.

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

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

Our success depends, in large part, on our ability to obtain and maintain patent protection in the U.S. and other countries with respect to our product candidates and our manufacturing technology. We and our licensors have sought to protect our proprietary position by filing patent applications in the U.S. and abroad related to many of our novel technologies and product candidates that are important to our business and may continue to do so.

The patent prosecution process is expensive, time-consuming and complex. Certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates may expire prior to commercial launch of our products; though we can mitigate this risk by pursuing and receiving 10 years Biologics regulatory exclusivity from the FDA, which would grant protection in later years where patent expiration may not exist. It is possible that we will fail to identify patentable aspects of our R&D output before it is too late to obtain patent protection, in part because the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates.

While we believe our intellectual property allows us to pursue our current development programs, several companies and academic institutions are pursuing alternate approaches to gene therapy and have built intellectual property around these approaches and methods. We may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Amendments) to conduct research and clinical trials and may be able to circumvent our patent rights by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patent rights may be challenged in the courts or patent offices in the U.S. and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of is technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide sufficient rights to exclude others from commercializing products similar or identical to ours.

If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.

We are party to intellectual property license agreements with several entities, each of which is important to our business, and we expect to enter into additional license agreements in the future. Our patent portfolio includes a number of patents and patent applications in-licensed pursuant to those license agreements, and those agreements impose, and we expect that future license agreements will impose various diligence, development and commercialization timelines, milestone obligations, payments and other obligations on us.

If we or our licensors breach any of the agreements under which we license intellectual property relating to the use, development and commercialization rights to our product candidates or technology from third parties, we could lose license rights that are important to our business. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of is product candidates;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- whether and the extent to which inventors are able to contest the assignment of their rights to our licensors.

If disputes over intellectual property that we have in-licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. In addition, if disputes arise as to ownership of licensed intellectual property, our ability to pursue or enforce the licensed patent rights may be jeopardized. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

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

In addition to the protection afforded by patents, we rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, employees and consultants. Nonetheless, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, however, despite the existence generally of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim that a third-party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing or unwilling to protect trade secrets.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. 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 our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

We may not be successful in obtaining or maintaining necessary rights to gene therapy product components and processes for our development pipeline through acquisitions and in-licenses.

We currently have intellectual property rights to develop our gene therapy product candidates, through third party licenses and our owned patents. Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently, and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies with greater cash resources and clinical development and commercialization capabilities are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive.

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

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

Obtaining and maintaining our 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 fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the U.S. in several stages over the lifetime of the patents and/or applications. We and, to our knowledge, our licensors have systems in place to remind us and them to pay these fees, and we and, to our knowledge, our licensors employ outside firms and rely on our and their respective outside counsel to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We and, to our knowledge, our licensors employ reputable law firms and other professionals to help us and them comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including patent eligible subject matter, lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our or our licensing partners' patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. Such prior art and prior art we have disclosed to the USPTO could impact the scope or validity of certain of our patent claims. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involves both technological and legal complexity, and therefore obtaining and enforcing biotechnology patents is costly, time-consuming, and inherently uncertain. Congress may pass patent reform legislation that is unfavorable to us.

The U.S. Supreme Court has ruled on several patent cases in recent years, narrowing the scope of patent protection available in certain circumstances and weakening the rights of patent owners in certain situations. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, recent court decisions raise questions regarding the award of patent term adjustment ("PTA") for patents in families where related patents have issued without PTA. Thus, it cannot be said with certainty how PTA will/will not be viewed in the future and whether patent expiration dates may be impacted.

Similarly, changes in patent laws and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or how they are enforced may weaken our ability to obtain new patents or to enforce patents that we have licensed or own in the future. For example, the complexity and uncertainty of European patent laws have also increased in recent years. In Europe, a new unitary patent system took effect June 1, 2023, which significantly impacts European patents, including those granted before the introduction of the system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent subject to the jurisdiction of the Unitary Patent Court ("UPC"). As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. Patents granted before the implementation of the UPC have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

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

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

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

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. 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 owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make product candidates that are similar to ours but that are not covered by the claims of the patents that we own;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or in-licensed intellectual property rights;
- our competitors might conduct R&D activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates;
- we cannot ensure that any patents issued to us, or our licensors will provide a basis for an exclusive market for our commercially viable product candidates or will provide us with any competitive advantages;
- we cannot ensure that our commercial activities or product candidates will not infringe upon the patents of others;
- we cannot ensure that we will be able to successfully commercialize our product candidates on a substantial scale, if approved, before the relevant patents that we own, or license expire;
- we may not develop additional proprietary technologies that are patentable;
- the patents or intellectual property rights of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We may have in the future, ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Risks Related to Personnel and Expansion of our Company

Risks Related to our Personnel

Our business could suffer if it loses the services of, or fails to attract, key personnel.

We are highly dependent upon the efforts of our senior management, including our Chief Executive Officer, Gaurav Shah, MD; our President and Chief Operating Officer, Kinnari Patel, PharmD, MBA; our Chief Business Officer and Senior Vice President, Raj Prabhakar; our Chief Medical Officer, Mark White, MB.ChB; our Vice President of Finance, Treasurer, Principal Accounting Officer and Interim Principal Financial Officer, John Militello; and our General Counsel, Chief Compliance Officer and Senior Vice President, Martin Wilson. The loss of the services of these individuals and other members of our senior management could delay or prevent the achievement of research, development, marketing, or product commercialization objectives. Our employment arrangements with the key personnel are “at-will.” We do not maintain any “key-man” insurance policies on any of the key employees nor do we intend to obtain such insurance. In addition, due to the specialized scientific nature of our business, we are highly dependent upon our ability to attract and retain qualified scientific and technical personnel and consultants. There is intense competition among major pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions for qualified personnel in the areas of our operations, and we may be unsuccessful in attracting and retaining these personnel.

Our employees, principal investigators, consultants, and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants, and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained during clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation or could cause regulatory agencies not to approve our product candidates. We have a code of business ethics and conduct applicable to all employees, but it is not always possible to identify and deter employee or third-party 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 comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we endeavor to ensure that our employees, consultants, and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Risks Related to Our Expansion and Growth Plans

We may need to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.

As our business activities expand, we may expand our full-time employee base and hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational setbacks, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected our ability to generate and/or grow revenues could be reduced and we may not be able to implement our business strategy.

We may fail to realize the anticipated benefits of potential acquisitions or business combinations.

The success of acquisitions or business combinations will depend on, among other things, our ability to combine our businesses in a manner that allows us to achieve developmental and operational synergies. It is possible that the integration process could result in the loss of key employees; the disruption of our ongoing business; or inconsistencies in standards, controls, procedures, or policies, in each case, that could adversely affect our ability to achieve the anticipated benefits of the acquisition. Integration efforts between the two businesses will also divert management's attention from our core business and other opportunities that could have been beneficial to our shareholders. An inability to realize the full extent of, or any of, the anticipated benefits of the acquisition, as well as any delays encountered in the integration process, could have an adverse effect on our business and results of operations, which may affect the value of the shares of our common stock after the completion of the acquisition. If we are unable to achieve these objectives, the anticipated benefits of the acquisition may not be realized fully or at all or may take longer or cost more to realize than expected. In particular, the acquisition or business combination may not be accretive to our stock value in the near or long term. In addition, any acquisition or business combination may impact the market price for shares of our common stock, which could result in substantial losses for our stockholders.

In addition, in connection with any potential acquisition of businesses, technologies or products in the future, we could, among other things:

- issue equity securities that would dilute our current stockholders' percentage ownership;
- incur substantial debt that may place strains on our operations;
- assume substantial actual or contingent liabilities;
- reprioritize our development programs and even cease development and commercialization of certain of our product candidates; or
- merge with, or otherwise enter into a business combination with, another company in which our stockholders would receive cash or shares of the other company on terms that certain of our stockholders may not deem desirable.

Although we intend to evaluate and consider acquisitions, reorganizations and business combinations in the future, we have no agreements or understandings with respect to any acquisition, reorganization or business combination at this time.

Future formations of strategic alliances or joint ventures with third parties could disrupt our business and harm our financial condition and operating results.

We may form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such strategic alliance or joint venture, we will achieve the expected synergies to justify the transaction. The risks we face in connection with any strategic alliance or joint venture, include:

- diversion of management time and focus from operating our business to addressing integration challenges;
- coordination of R&D efforts;
- changes in relationships with strategic partners as a result of any product acquisitions or strategic positioning;
- cultural challenges associated with integrating employees;
- the need to implement or improve controls, procedures, and policies at any joint venture;
- liability for activities of any partnered company prior to any strategic alliance or joint venture, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims, including claims from employees, customers, former stockholders or other third parties

Our failure to address these risks or other problems encountered in connection with our past or future strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future strategic alliances or joint ventures could result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or operating results.

Given our commercial relationships outside of the U.S., in particular in the EU, a variety of risks associated with international operations could harm our business.

We engage in various commercial relationships outside the U.S., and we may commercialize our product candidates outside of the U.S. In many foreign countries, it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the Foreign Corrupt Practices Act. Although we may implement policies and procedures specifically designed to comply with these laws and policies, there can be no assurance that our employees, contractors, and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

We may be, and to the extent we commercialize our product candidates outside the U.S., expect to be subject to various risks associated with operating internationally, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- 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;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;

- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, public health crises such as pandemics and epidemics, or from economic or political instability;
- compliance with foreign laws, regulations, standards, and regulatory guidance governing the collection, use, disclosure, retention, security and transfer of personal data, including the GDPR and UK GDPR; and
- greater difficulty with enforcing our contracts in jurisdictions outside of the U.S.

These and related risks could materially harm our business, financial condition, results of operations and prospects.

If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.

If conflicts arise between our corporate or academic collaborators or strategic partners and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Some of our academic collaborators and strategic partners are conducting multiple product development efforts within each area that is the subject of the collaboration with us. Our collaborators or strategic partners, however, may develop, either alone or with others, products in related fields that are competitive with the products or potential products that are the subject of these collaborations. Competing products, either developed by the collaborators or strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of our collaborator's or partner's support for our product candidates.

Some of our collaborators or strategic partners could also become our competitors in the future. Our collaborators or strategic partners could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, or fail to devote sufficient resources to the development and commercialization of our product candidates. Any of these developments could harm our product development efforts.

Risks Related to Ownership of our Common Stock

Future sales of our common stock in the public market could cause the market price of our common stock to drop significantly, even if our business is performing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception or the perception that such sales may occur, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended (the "Securities Act"), or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates of ours. In addition, certain of our employees, executive officers, directors, and affiliated stockholders may enter into Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the employee, director or officer when entering into the plan, without further direction from the employee, officer, director or affiliated stockholder. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers, directors and affiliated stockholders also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information. In addition, in the future, we may issue additional shares of common stock or other equity or debt securities convertible into common stock in connection with a financing, acquisition, litigation settlement, employee arrangements or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. Although we have obtained analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock or fail to regularly publish reports on us, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for our stockholders.

Our stock price is likely to be volatile. The stock market in general, and the market for biopharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the U.S. and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- negative publicity around gene therapy in general, or our product candidates;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- macroeconomic conditions, including inflation and rising interest rates, capital market volatility and global conflicts, including the Russia-Ukraine war, the Israel-Hamas war and the conflict between China and Taiwan;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

RTW Investments, LP, our largest stockholder, may have the ability to significantly influence all matters submitted to stockholders for approval.

RTW Investments, LP ("RTW"), in the aggregate, beneficially owns approximately 20.21% of our outstanding shares of common stock. This concentration of voting power gives RTW the power to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, RTW could significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. In addition, this may prevent or discourage unsolicited acquisition proposals or offers for our capital stock that you may believe are in your best interest as one of our stockholders.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

General Risk Factors

Our limited operating history may make it difficult for us to evaluate the success of our business to date and to assess our future viability.

Our operations to date have predominantly focused on organizing and staffing our company, business planning, raising capital, acquiring our technology, administering, and expanding our gene therapy platforms, identifying potential product candidates, undertaking research, preclinical studies and clinical trials of our product candidates, building out our R&D and manufacturing capabilities, and establishing licensing arrangements and collaborations. We have not yet obtained marketing approvals, manufactured a commercial-scale product, or conducted sales and marketing activities necessary for successful commercialization. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history. We are currently a drug discovery and clinical stage company and at a later point we will need to transition to a commercial stage company. We cannot guarantee that we will be successful in this transition.

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, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.

As a public company, we are required to maintain internal control over financial reporting and to report any material weaknesses in such internal control. Pursuant to Section 404 of the Sarbanes-Oxley Act ("Section 404"), we are required to furnish a report by management on the effectiveness of our internal control over financial reporting and our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting. Preparing such attestation report and the cost of compliance with reporting requirements requires significant management time.

The rules governing the standards that must be met for management and our independent registered public accounting firm to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation. In connection with our and our independent registered public accounting firm's evaluations of our internal control over financial reporting, we may need to upgrade systems, including information technology, implement additional financial and management controls, reporting systems, and procedures, and hire additional accounting and finance staff.

Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us or our independent registered public accounting firm conducted in connection with Section 404 may reveal deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. We could become subject to stockholder or other third-party litigation, as well as investigations by the SEC, NASDAQ or other regulatory authorities, which could require additional financial and management resources and could result in fines, trading suspensions, payment of damages or other remedies. Further, any delay in compliance with the auditor attestation provisions of Section 404 could subject us to a variety of administrative sanctions, including ineligibility for short-form resale registration, action by the SEC and the suspension or delisting of our common stock, which could reduce the trading price of our common stock and could harm our business.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our Board of Directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our Board of Directors. Among other things, these provisions:

- permit only the Board of Directors to establish the number of directors;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders; and
- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

Moreover, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. Any of these provisions of our charter documents or Delaware law could, under certain circumstances, depress the market price of our common stock.

Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Our internal computer systems and those of our current and any future collaborators and other consultants and contractors are vulnerable to damage from computer viruses, unauthorized access, cyberattacks, data breaches, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident, attack or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security 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, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed.

Unfavorable national or global economic conditions or political developments could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the national or global economy and financial markets. For example, governmental statements, actions or policies, political unrest and global financial crises can cause extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, political unrest or additional global financial crises, including those resulting from the COVID-19 pandemic and the ongoing Russia-Ukraine war, Israel-Hamas war and the conflict between China and Taiwan, could result in a variety of risks to our business, including weakened demand for our products, if approved, or our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate, further political developments and financial market conditions could adversely impact our business.

The outbreak of SARS-CoV-2, which causes COVID-19, or other similar pandemics in the future could adversely impact our business, including our preclinical and clinical studies.

As a result of the ongoing COVID-19 outbreak, or similar pandemics, we have and may in the future experience disruptions that could severely impact our business, preclinical studies, and clinical trials, including:

- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources from the conduct of clinical trials such as patient follow up visits, the diversion of hospitals ability to serve as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- delays or difficulties in securing manufacturing slots or materials;
- delays or difficulties in advancing preclinical research requiring in-person laboratory work at our facility at academic partners or contract research facilities; and
- interruption or delays in the operations of the FDA and/or comparable foreign regulatory agencies, which may impact approval timelines.

Item 1B. Unresolved SEC Comments

None.

Item 1C. Cybersecurity

The Company maintains a cybersecurity risk management program designed to identify, assess, manage, mitigate, and respond to cybersecurity threats. The program is integrated within the Company's enterprise risk management framework and addresses both the corporate information technology environment and the external facing ecosystem.

The underlying controls of the cybersecurity risk management program are based on recognized best practices and standards for cybersecurity and information technology, including the National Institute of Standards and Technology ("NIST") Cybersecurity Framework ("CSF") and the International Organization for Standardization ("ISO") 27001 Information Security Management System Requirements. The Company will have a third party perform an annual assessment of the Company's cybersecurity risk management program against the NIST CSF. The Company has a Cyber Security Operations Center monitoring our global cybersecurity environment and coordinates investigations and remediation of alerts. We are enhancing our programs for staging incident response drills to prepare support teams in the event of a significant incident. The cybersecurity risk management program includes controls for organizational processes, personnel, physical facilities and equipment, and technological controls.

Our VP, Head of Information Technology is the Company's designated Chief Information Security Officer ("CISO") and is responsible for developing and implementing the cybersecurity risk management program and reporting on cybersecurity matters to the Board. The VP, Head of Information Technology has over twenty years of experience leading cybersecurity oversight. Additionally, members of the IT security team have cybersecurity experience and\or certifications, such as the Certified Information Systems Security Professional certification and Certified Information Systems Audit certification. We view cybersecurity as a shared responsibility across our management team, and plan to periodically perform simulations and tabletop exercises at a management level and incorporate external resources and advisors as needed. All employees will be required to complete cybersecurity training at least once annually and have access to more frequent cybersecurity training through online and live events. We also require employees in certain roles to complete additional role-based, specialized cybersecurity training that is documented in our quality management system. Employees outside of our corporate information security organization also have a role in our cybersecurity defenses and they are immersed in a corporate culture supportive of security, which we believe improves our cybersecurity.

Our CISO is responsible for continuously monitoring and assessing the Company's cybersecurity risk management program, informing senior management regarding the prevention, detection, mitigation, and remediation of cybersecurity incidents and supervising such efforts. The cybersecurity team collectively has decades of experience selecting, deploying, and operating cybersecurity technologies, initiatives, and processes around the world, and relies on threat intelligence as well as information obtained from governmental, public, and private sources, including external consultants engaged by the Company on a real time basis. The Company is enhancing its processes for oversight of third-party vendors, including appropriate due diligence for new providers and continuous monitoring following implementation, including ongoing direct contact with vendor personnel. Third-party vendors are re-evaluated at regular intervals as part of our supplier qualification process.

The Audit Committee, in addition to the Company's General Counsel and Chief Compliance Officer, oversees the Company's cybersecurity risk exposures and the steps taken by management to monitor and mitigate cybersecurity risks. The cybersecurity team briefs the Audit Committee and General Counsel and Chief Compliance Officer on the effectiveness of the Company's cyber risk management program, generally on a quarterly basis. In addition, cybersecurity risks will be reviewed by the Board of Directors, at least annually, as part of the Company's corporate risk mapping exercise.

We have not experienced any material cybersecurity incidents in the past, and we believe no cybersecurity events have occurred that have materially affected the Company or its business strategy, results of operations or financial condition. We continue to invest in the cybersecurity and resiliency of our infrastructure and the enhancement of our internal controls and processes, which are designed to help protect our systems and data, and the information they contain. For more information regarding the risks we face from cybersecurity threats, please see "Risk Factors."

Item 2. Properties

Corporate Headquarters, R&D and GMP Manufacturing Facility, Storage Facility

Rocket's corporate headquarters is located in Cranbury, New Jersey, in a leased facility consisting of 103,720 square feet of space including areas for offices, process development, research and development laboratories and 50,000 square feet dedicated to AAV cGMP manufacturing to support our pipeline. The NJ Lease Agreement has an initial term ending in 2034, with an option to renew for an additional two consecutive five-year renewal terms. In addition, we lease space in New York, New York at the Empire State Building, which consists of approximately 6,600 square feet of office space under a lease that expires in July 2024. Rocket leases an additional 4,666 square feet storage facility in Dayton, New Jersey.

Facilities in Hopewell, New Jersey

As part of the acquisition of Renovacor, we assumed lease agreements for approximately 15,463 square feet of space in Hopewell, New Jersey that expires in March 2033. The Company intends to sublease these facilities and signed a sublease agreement for one of the Hopewell, NJ facilities in January 2024.

Facility in Cambridge, Massachusetts

As part of the acquisition of Renovacor, we assumed a sublease agreement for approximately 5,945 square feet of office space in Cambridge, Massachusetts that expires in April 2024.

Item 3. Legal Proceedings

From time to time, we may be subject to other 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, we do not believe we are party to any other 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.

Item 4. Mine Safety Disclosures

Not Applicable.

PART II

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

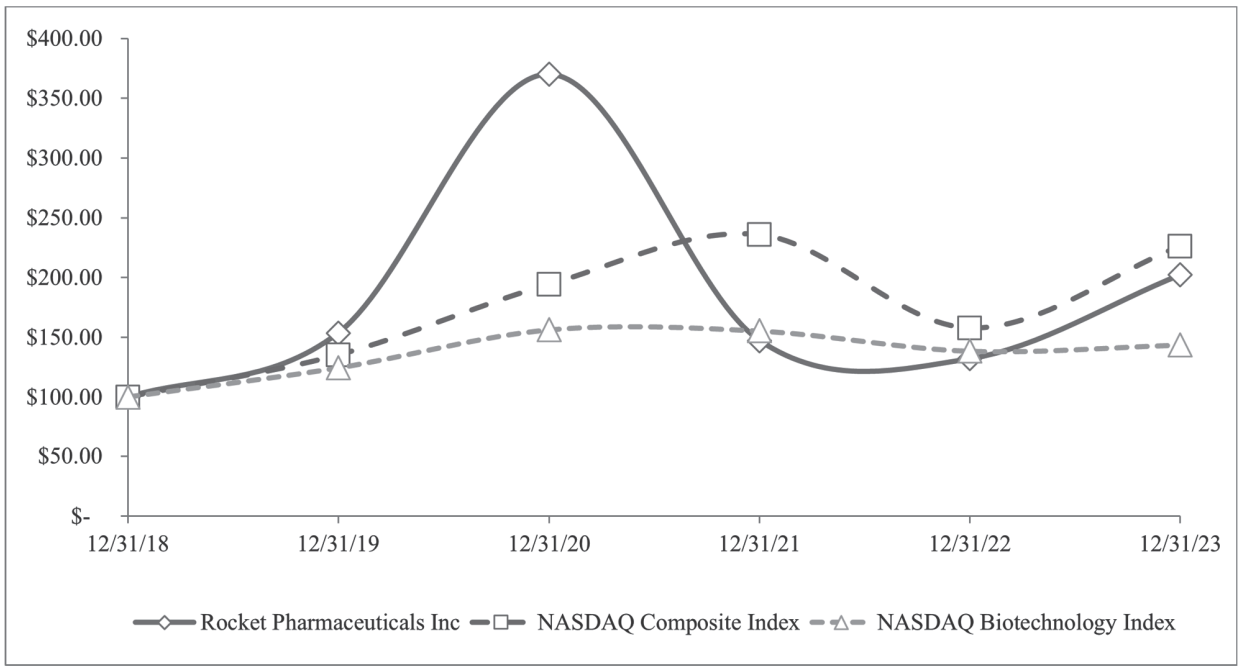
Market Information

Our common stock is traded on the NASDAQ Global Market under the symbol "RCKT". On February 22, 2024, the last reported sale price for our common stock on the Nasdaq Global Market was $29.00 per share.

Stock Performance Graph

The graph set forth below compares the cumulative total stockholder return on our common stock between January 1, 2019 and December 31, 2023 with the cumulative total return of (a) the NASDAQ Biotechnology Index and (b) the NASDAQ Composite Index, over the same period. This graph assumes the investment of $100 on January 1, 2019 of our common stock, the NASDAQ Biotechnology Index and the NASDAQ Composite Index and assumes the reinvestment of dividends, if any.

The comparisons shown in the graph below are based upon historical data. We caution that the stock price performance shown in the graph below is not necessarily indicative of, nor is it intended to forecast, the potential future performance of our common stock.



Stockholders

As of February 22, 2024, there were 32 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay cash dividends will be made at the discretion of our Board of Directors. Investors should not purchase our common stock with the expectation of receiving cash dividends.

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 on Form 10-K.

Recent Sales of Unregistered Securities

None.

Use of Proceeds from Public Offering of Common Stock

On September 15, 2023, we completed a Public Offering of approximately 9.5 million shares of our common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant ("September 2023 Public Offering"). The gross proceeds from the September 2023 Public Offering were approximately $201.3 million, net of $12.4 million of offering costs, underwriting discounts and commissions, legal and other expenses for net proceeds from the offering of $188.9 million. The offer and sale of the shares and pre-funded warrants were registered under the Securities Act pursuant to a prospectus supplement, filed with the SEC on September 15, 2023, to the Company's effective registration statement on Form S-3 (Registration No. 333-253756), which was previously filed with the SEC, and declared effective on September 10, 2021. There has been no material change in the planned use of proceeds from our September 2023 Public Offering as described in the prospectus supplement related to the offering.

Issuer Purchases of Equity Securities

There were no repurchases of our common stock during the year ended December 31, 2023.

Item 6. Reserved

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements, related notes and other financial information included elsewhere in this Annual Report. This discussion contains forward-looking statements that involve risks and uncertainties such as our plans, objectives, expectations, and intentions. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in "Risk Factors" included elsewhere in this Annual Report.

Unless otherwise indicated, references to "*Rocket,*" *the* "*Company,*" "*we,*" "*our*" *and* "*us*" *refer to Rocket Pharmaceuticals, Inc. and its subsidiaries.*

Introduction

We are a fully integrated, late-stage biotechnology company focused on the development of first, only and best in class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. We have three clinical-stage *ex vivo* lentiviral vector ("LV") programs, which include programs for:

- Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells;
- Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction; and
- Pyruvate Kinase Deficiency ("PKD"), a red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia.

In September 2023, the FDA accepted the Biologics License Application ("BLA") and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 studies were completed in 2023 with regulatory filings in the United States ("U.S.") and Europe ("EU") for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., we also have two clinical stage and one pre-clinical stage *in vivo* adeno-associated virus ("AAV") programs, which include programs for:

- Danon disease ("DD"), a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The DD program is currently in an ongoing Phase 2 trial.
- Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), an inheritable cardiac disorder that is characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. This program received FDA clearance of an Investigational New Drug ("IND") application and we have initiated a Phase 1 study.
- BAG3 Dilated Cardiomyopathy ("DCM"), which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. Our program utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Recent Developments

At-the-Market Offering Program

On February 28, 2022, we entered into a Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which we may offer and sell, from time to time at our sole discretion, shares through Cowen as our sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to our shelf registration statement on Form S-3. We filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. We will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. We also agreed to provide Cowen with customary indemnification and contribution rights. We have reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through December 31, 2023, we sold 4.2 million shares under the at-the-market offering program for gross proceeds of $65.8 million, less commissions of $2.0 million for net proceeds of $63.8 million. During the year ended December 31, 2023, we sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commissions of approximately $0.6 million for net proceeds of $17.2 million. On September 12, 2023, the Company and Cowen entered into an amendment pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million.

Public Offering

On September 15, 2023, we completed a public offering of approximately 9.5 million shares of our common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant (the "September 2023 Public Offering"). The gross proceeds from the September 2023 Public Offering were approximately $201.3 million, net of $12.4 million of offering costs, underwriting discounts and commissions, legal and other expenses for net proceeds from the offering of $188.9 million.

Financial Overview

Since our inception, we have devoted substantially all of our resources to organizing and staffing the Company, business planning, raising capital, acquiring, or discovering product candidates and securing related intellectual property rights, conducting discovery, R&D activities for our product candidates and planning for potential commercialization. We do not have any products approved for sale and have not generated any revenue from product sales. From inception through December 31, 2023, we raised net cash proceeds of approximately $1.0 billion from investors through both equity and convertible debt financing to fund operating activities.

Revenue

To date, we have not generated any revenue from any sources, including from product sales, and we do not expect to generate any revenue from the sale of products in the near future. If our development efforts for product candidates are successful and result in regulatory approval or license agreements with third parties, we may generate revenue in the future from product sales.

Research and Development Expenses

Our R&D program expenses consist primarily of external costs incurred for the development of our product candidates. These expenses include:

- expenses incurred under agreements with research institutions and consultants that conduct R&D activities, including process development and preclinical and clinical activities on our behalf;
- costs related to process development and production of preclinical and clinical materials, including fees paid to contract manufacturers and manufacturing input costs for use in internal manufacturing processes;
- consultants supporting process development and regulatory activities; and
- costs related to in-licensing of rights to develop and commercialize our product candidate portfolio.

We recognize external development costs based on contractual payment schedules aligned with program activities, invoices for work incurred, and milestones that correspond with costs incurred by the third parties. Nonrefundable advance payments for goods or services to be received in the future for use in R&D activities are recorded as prepaid expenses.

Our direct R&D expenses are tracked on a program-by-program basis for product candidates and consist primarily of external costs, such as research collaborations and third-party manufacturing agreements associated with our preclinical research, process development, manufacturing, and clinical development activities. Our direct R&D expenses by program also include fees incurred under license agreements. Our personnel, non-program and unallocated program expenses include costs associated with activities performed by our internal R&D organization and generally benefit multiple programs. These costs are not separately allocated by product candidate and consist primarily of:

- salaries and personnel-related costs, including benefits, travel, and stock-based compensation, for our scientific personnel performing R&D activities;
- facilities and other expenses, which include expenses for rent and maintenance of facilities, and depreciation expense; and
- laboratory supplies and equipment used for internal R&D activities.

We allocate salary and benefit costs directly related to specific programs. We do not allocate personnel-related discretionary bonus or stock-based compensation costs, costs associated with our general discovery platform improvements, depreciation or other indirect costs that are deployed across multiple projects under development and, as such, the costs are separately classified as other R&D expenses.

The following table presents R&D expenses, in thousands, tracked on a program-by-program basis as well as by type and nature of our expense for our product candidates for the years ended December 31, 2023 and 2022, and 2021.

	Years Ended December 31,		
	2023	2022	2021
Direct Expenses:			
Danon Disease (AAV) RP-A501	$ 28,992	$ 28,524	$ 15,804
Plakophilin-2 Arrhythmogenic Cardiomyopathy (AAV) RP-A601	7,171	11,724	1,071
Leukocyte Adhesion Deficiency (LVV) RP-L201	17,725	20,617	24,222
Fanconi Anemia (LVV) RP-L102	25,276	23,917	15,453
Pyruvate Kinase Deficiency (LVV) RP-L301	4,808	2,744	4,206
Infantile Malignant Osteopetrosis (LVV) RP-L401 (1)	-	271	2,236
Other product candidates	5,501	3,580	3,504
Total direct expenses	89,473	91,377	66,496
Unallocated Expenses:			
Employee compensation	$ 46,867	$ 32,274	$ 20,780
Non-cash R&D expense related to the issuance of warrants	-	-	12,781
Stock based compensation expense	17,509	12,465	11,954
Depreciation and amortization expense	5,375	4,037	5,130
Laboratory and related expenses	17,618	17,405	3,359
Professional fees	3,927	3,601	1,797
Other expenses	5,573	4,411	3,179
Total other research and development expenses	96,869	74,193	58,980
Total research and development expense	$ 186,342	$ 165,570	$ 125,476

(1) Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. Costs to close out the study were incurred in 2022.

We cannot determine with certainty the duration and costs to complete current or future clinical studies of product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical studies and development of product candidates will depend on a variety of factors, including:

- the scope, rate of progress, and expense of ongoing clinical studies as well as any clinical studies and other R&D activities that we undertake in the future;
- future clinical study results;
- uncertainties in clinical study enrollment rates;
- changing standards for regulatory approval; and
- the timing and receipt of any regulatory approvals.

We expect R&D expenses to increase for the foreseeable future as we continue to invest in R&D activities related to developing product candidates, including investments in manufacturing, as our programs advance into later stages of development and as we conduct additional clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of R&D projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Our future R&D expenses will depend on the clinical success of our product candidates, as well as ongoing assessments of the commercial potential of such product candidates. In addition, we cannot forecast with any degree of certainty which product candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements. We expect our R&D expenses to increase for the foreseeable future as we seek further development of our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the scope, progress, outcome and costs of our clinical trials and other R&D activities;
- the efficacy and potential advantages of our product candidates compared to alternative treatments, including any standard of care;
- the market acceptance of our product candidates;
- obtaining, maintaining, defending, and enforcing patent claims and other intellectual property rights;
- significant and changing government regulation; and
- the timing, receipt, and terms of any marketing approvals.

A change in the outcome of any of these variables with respect to the development of our product candidates that we may develop could mean a significant change in the costs and timing associated with the development of our product candidates. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials or other testing beyond those that we currently contemplate for the completion of clinical development of any of our product candidates that we may develop or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefit costs for personnel, including stock-based compensation and travel expenses for our employees in commercial, executive, operational, finance, legal, business development, and human resource functions. In addition, other significant general and administrative expenses include professional fees for legal, consulting, investor and public relations, auditing, and tax services as well as other expenses for rent and maintenance of facilities, insurance and other supplies used in general and administrative activities. We expect general and administrative expenses to increase for the foreseeable future due to anticipated increases in headcount to support the continued advancement of our product candidates and our progression to commercial operations. We also anticipate that as we continue to operate as a public company with increasing complexity, we will continue to incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses.

Interest Expense

Interest expense in 2023 and 2022 was related to our financing lease obligation for our Cranbury, NJ facility. Interest expense in 2021 was related to convertible notes due in 2021, which were converted into common stock in August 2021 and convertible notes due in 2022, which were converted into common stock in April 2021, and our financing lease obligation for the Cranbury, NJ facility.

Interest and Other Income

Interest and other income related to interest earned from investments and cash equivalents, liability extinguishment and reduced fair value of warrant liability.

Results of Operations

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations, in thousands, for each of the periods presented:

	For the Years Ended December 31,		
	2023	2022	Change
Operating expenses:			
Research and development	$ 186,342	$ 165,570	$ 20,772
General and administrative	73,317	58,773	14,544
Total operating expenses	259,659	224,343	35,316
Loss from operations	(259,659)	(224,343)	(35,316)
Research and development incentives	-	500	(500)
Interest expense	(1,875)	(1,862)	(13)
Interest and other income, net	5,288	3,889	1,399
Accretion of discount and amortization of premium on investments, net	10,651	(47)	10,698
Total other income, net	14,064	2,480	11,584
Net loss	$ (245,595)	$ (221,863)	$ (23,732)

Research and Development Expenses

R&D expenses increased $20.8 million to $186.3 million for the year ended December 31, 2023, compared to the year ended December 31, 2022. The increase in R&D expenses was primarily driven by increases in costs for compensation and benefits of $16.9 million due to increased R&D headcount, clinical trial costs of $14.5 million, non-cash stock compensation expense of $5.0 million, and license expenses of $2.2 million. Increases noted were partially offset by decreases in manufacturing and development costs of $17.0 million and direct materials of $3.5 million.

General and Administrative Expenses

G&A expenses increased $14.5 million to $73.3 million for the year ended December 31, 2023, compared to the year ended December 31, 2022. The increase in G&A expenses was primarily driven by increases in commercial preparation related expenses of $8.4 million, non-cash stock compensation expense of $3.4 million, and legal expenses of $3.0 million, which were partially offset by a reduction in acquisition related expenses of $3.0 million due to the closing of the Renovacor acquisition in 2022.

Other Income, Net

Other income, net increased by $11.6 million to $14.1 million for the year ended December 31, 2023, compared to the year ended December 31, 2022. The increase in other income was primarily driven by an increase in accretion of discount and amortization of premium on investments, net, of $10.7 million and interest and other income, net, of $1.4 million. The increase in interest and other income, net, of $1.4 million was due to increased interest rates of $1.6 million and a liability extinguishment of $0.6 million, partially offset by increased fair value of warrant liability of $0.4 million.

Comparison of the Years Ended December 31, 2022 and 2021

The following table summarizes our results of operations, in thousands, for each of the periods presented:

	For the Years Ended December 31, 2022	2021	Change
Operating expenses:			
Research and development	$ 165,570	$ 125,476	$ 40,094
General and administrative	58,773	41,772	17,001
Total operating expenses	224,343	167,248	57,095
Loss from operations	(224,343)	(167,248)	(57,095)
Research and development incentives	500	1,000	(500)
Interest expense	(1,862)	(2,977)	1,115
Interest and other income, net	3,889	3,068	821
Accretion of discount and amortization of premium on investments, net	(47)	(2,912)	2,865
Total other income (expense), net	2,480	(1,821)	4,301
Net loss	$ (221,863)	$ (169,069)	$ (52,794)

Research and Development Expenses

R&D expenses increased $40.1 million to $165.6 million for the year ended December 31, 2022, compared to the year ended December 31, 2021. The increase in R&D expenses was primarily driven by increases in manufacturing and development costs of $26.3 million, laboratory supplies of $6.6 million, compensation and benefits expense of $11.5 million due to increased R&D headcount, direct materials of $3.6 million, and consulting and professional fees of $2.7 million. Increases noted were offset by a decrease in license fees of $12.9 million attributable to the expense in connection with warrants to purchase shares of common stock recorded for the year ended December 31, 2021.

General and Administrative Expenses

G&A expenses increased $17.0 million to $58.8 million for the year ended December 31, 2022, compared to the year ended December 31, 2021. The increase in G&A expenses was primarily driven by increases in commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $4.9 million, compensation and benefits of $4.4 million due to increased G&A headcount and acquisition related expenses of $3.2 million.

Other Income, Net

Other income, net increased by $4.3 million to $2.5 million for the year ended December 31, 2022, compared to the year ended December 31, 2021. The change was primarily driven by decreased interest expense of $1.1 million associated with convertible notes due in 2022, which were converted into common stock in April 2021 and convertible notes due in 2021, which were converted into common stock in August 2021, an increase in interest and other income, net, of $0.8 million due to increased interest rates and a decrease in amortization of premium on investment, net, of $2.9 million.

Liquidity and Capital Resources

We have not generated any revenue and have incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, those related to drug candidate development, technology and data security, patents and proprietary rights, our lack of commercial manufacturing marketing or sales experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional R&D efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

Our drug candidates are in the development and clinical stage. There can be no assurance that our R&D will be successfully completed, that adequate protection for our intellectual property will be obtained, that any products developed will obtain necessary government approval or that any approved products will be commercially viable. Even if our product development efforts are successful, it is uncertain when, if ever, we will generate significant revenue from product sales. We operate in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

Our consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. Rocket has incurred net losses and negative cash flows from its operations each year since inception. Rocket incurred net losses of $245.6 million, $221.9 million, and $169.1 million for the years ended December 31, 2023, 2022 and 2021, respectively. We have experienced negative cash flows from operations and have an accumulated deficit of $959.4 million as of December 31, 2023. As of December 31, 2023, we had $407.5 million of cash, cash equivalents and investments. Included in the $407.5 million cash balance are securities that have yet to be paid of $13.1 million related to investments in securities that settled in 2024. The net cash balance, when adjusting for this payable would have been $394.4 million. We expect such resources would be sufficient to fund our operating expenses and capital expenditure requirements into 2026. We have funded our operations primarily through the sale of equity.

On April 30, 2019, CIRM awarded the Company up to $7.5 million under a CLIN2 grant award program to support the clinical development of our LV-based gene therapy for RP-L201 based on achievements of specific development milestones. The Company achieved two milestones in 2019 and received $1.1 million. In 2020, the Company achieved two more milestones and received $2.8 million. The Company achieved an additional milestone in 2021 and received $1.0 million. In 2022, the Company achieved one more milestone and received $0.9 million. In 2023, the Company achieved a final milestone resulting in a payment of $0.05 million. No additional payments are available under the grant award program as of December 31, 2023.

In the longer term, our future viability is dependent on our ability to generate cash from operating activities or to raise additional capital to finance our operations. If we raise additional funds by issuing equity securities, our stockholders will experience dilution. Any future debt financing into which we enter may impose upon us additional covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, repurchase our common stock, make certain investments and engage in certain merger, consolidation, or asset sale transactions. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our failure to raise capital as and when needed could have a negative impact on our financial condition and ability to pursue our business strategies.

At-the-Market Offering Program

On February 28, 2022, we entered into a Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which we may offer and sell, from time to time at our sole discretion, shares through Cowen as our sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to our shelf registration statement on Form S-3. We filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. We will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. We also agreed to provide Cowen with customary indemnification and contribution rights. We have reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through December 31, 2023, we sold 4.2 million shares under the at-the-market offering program for gross proceeds of $65.8 million, less commissions of $2.0 million for net proceeds of $63.8 million. During the year ended December 31, 2023, we sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commissions of approximately $0.6 million for net proceeds of $17.2 million. On September 12, 2023, the Company and Cowen entered into an amendment pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million.

Public Offering

On September 15, 2023, we completed a public offering of approximately 9.5 million shares of our common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant (the "September 2023 Public Offering"). The gross proceeds from the September 2023 Public Offering were approximately $201.3 million, net of $12.4 million of offering costs, underwriting discounts and commissions, legal and other expenses for net proceeds from the offering of $188.9 million.

Contractual Obligations

In the normal course of business, we enter into contracts and commitments that obligate us to make payments in the future. Information regarding our obligations relating to income taxes and lease arrangements are provided in "Note 12. Income Taxes" and "Note 13. Leases" to our consolidated financial statements contained in "Item 8. Financial Statements and Supplementary Data."

We do not have any off-balance sheet arrangements that are material or reasonably likely to become material to our financial condition or results of operations.

Cash Flows

The following table summarizes our cash flows from operating, investing and financing activities, in thousands, for each of the periods presented:

	For the Years Ended December 31,		
	2023	2022	2021
Net cash used in operating activities	$ (194,916)	$ (178,142)	$ (121,163)
Net cash (used in) provided by investing activities	(98,066)	(69,326)	18,853
Net cash provided by financing activities	208,401	155,288	37,681
Net decrease in cash, cash equivalents and restricted cash	$ (84,581)	$ (92,180)	$ (64,629)

Operating Activities

During the year ended December 31, 2023, operating activities used $194.9 million of cash and cash equivalents, primarily resulting from our net loss of $245.6 million offset by net non-cash charges of $37.2 million, including non-cash stock-based compensation expense of $39.4 million, depreciation and amortization expense of $7.1 million, impairment of acquired intangible asset and write down of property and equipment of $0.9 million, partially offset by accretion of discount on investments of $10.2 million. Changes in our operating assets and liabilities for the year ended December 31, 2023 included an increase in accounts payable and accrued expenses of $10.1 million, and a decrease in our prepaid expenses and other assets of $2.7 million.

During the year ended December 31, 2022, operating activities used $178.1 million of cash and cash equivalents, primarily resulting from our net loss of $221.9 million and net changes in our operating assets and liabilities of $6.1 million, partially offset by net non-cash charges of $37.6 million, including stock-based compensation expense of $31.0 million and depreciation and amortization expense of $6.3 million. Changes in our operating assets and liabilities for the year ended December 31, 2022 consisted of an increase in accounts payable and accrued expenses of $9.7 million and an increase in prepaid expenses and other assets of $3.6 million.

During the year ended December 31, 2021, operating activities used $121.2 million of cash and cash equivalents, primarily resulting from our net loss of $169.1 million and net changes in our operating assets and liabilities of $3.4 million, partially offset by net non-cash charges of $51.3 million, including expenses in connection with the issuance of warrant of $12.8 million, stock-based compensation expense of $29.2 million, amortization of premium on investments of $2.9 million and depreciation and amortization expense of $5.4 million. Changes in our operating assets and liabilities for the year ended December 31, 2021 consisted of a decrease in accounts payable and accrued expenses of $4.8 million and a decrease in prepaid expenses and other assets of $1.3 million.

Investing Activities

During the year ended December 31, 2023, net cash used in investing activities was $98.1 million, primarily resulting from proceeds of $309.3 million from the maturities of investments, offset by purchases of investments of $390.9 million, and purchases of property and equipment of $16.5 million.

During the year ended December 31, 2022, net cash used in investing activities was $69.3 million, consisting of proceeds of $272.9 million from the maturities of investments and proceeds of $42.7 million from the acquisition of Renovacor, offset by purchases of investments of $376.3 million, purchases of property and equipment of $8.4 million and purchases of right of use assets of $0.3 million.

During the year ended December 31, 2021, net cash provided by investing activities was $18.9 million, consisting of proceeds of $272.4 million from the maturities of investments, offset by purchases of investments of $245.9 million, and purchases of property and equipment of $7.6 million.

Financing Activities

During year ended December 31, 2023, net cash provided by financing activities was $208.4 million, consisting primarily of proceeds related to the September 2023 Public Offering of $188.9 million, $17.2 million from issuance of common stock through our at-the-market offering program and $2.2 million from the exercise of stock options.

During the year ended December 31, 2022, net cash provided by financing activities was $155.3 million, consisting primarily of proceeds related to the October 2022 Public Offering of $108.1 million and issuance of common stock through our at-the-market offering program of $46.6 million.

During the year ended December 31, 2021, net cash provided by financing activities was $37.7 million, consisting of proceeds from the issuance of common stock related to a private placement in August 2021 of $26.4 million and issuance of common stock, pursuant to exercises of stock options, of $11.3 million.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in conformity with accounting principles generally accepted in the U.S. ("U.S. GAAP"). The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We periodically review our estimates as a result of changes in circumstances, facts and experience. The effects of material revisions in estimates are reflected in the financial statements prospectively from the date of the change in estimate.

For a description of our significant accounting policies, refer to "Note 3. Summary of Significant Accounting Policies" included in the notes to our consolidated financial statements appearing elsewhere in this report. We consider the most critical accounting policies to be those related to our Accrued R&D Expenses, Stock-Based-Compensation, Goodwill and Intangible Assets.

Goodwill

Business combinations are accounted for under the acquisition method. The total cost of an acquisition is allocated to the underlying identifiable net assets, based on their respective estimated fair values as of the acquisition date. Determining the fair value of assets acquired and liabilities assumed requires management's judgment and often involves the use of significant estimates and assumptions, including assumptions with respect to future cash inflows and outflows, discount rates, asset lives and market multiples, among other items. Assets acquired and liabilities assumed are recorded at their estimated fair values. The excess of the purchase price over the estimated fair values of the net assets acquired is recorded as goodwill. Goodwill is tested for impairment annually as of December 31, or more frequently when events or changes in circumstances indicate that the asset might be impaired. Examples of such events or circumstances include, but are not limited to, a significant adverse change in legal or business climate, an adverse regulatory action or unanticipated competition. The Company has one segment and one reporting unit and as such reviews goodwill for impairment at the consolidated level. When testing goodwill, the Company has the option to first assess qualitative factors for reporting units that carry goodwill. The qualitative assessment includes assessing the totality of relevant events and circumstances that affect the fair value or carrying value of the reporting unit. These events and circumstances include macroeconomic conditions, industry and competitive environment conditions, overall financial performance, reporting unit specific events and market considerations. The Company also considers recent valuations of the reporting unit, including the magnitude of the difference between the most recent fair value estimate and the carrying value, as well as both positive and adverse events and circumstances, and the extent to which each of the events and circumstances identified may affect the comparison of a reporting unit's fair value with its carrying value. If the qualitative assessment results in a conclusion that it is more likely than not that the fair value of a reporting unit exceeds the carrying value, then no further testing is performed for that reporting unit.

The Company performed the qualitative assessment of its goodwill and determined that it is more likely than not that the fair value of a reporting unit exceeds the carrying value of the reporting unit. As a result, the Company has determined there was no goodwill impairment as of and for the years ended December 31, 2023, 2022 and 2021.

Intangible Assets

Intangible assets consisted of indefinite lived intangible in process research and development ("IPR&D") assets and a mice colony model. Intangible assets related to IPR&D projects are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time. IPR&D intangible assets which are determined to have had a drop in their fair value are adjusted downward and an expense recognized in R&D expenses in the Consolidated Statements of Operations. These IPR&D intangible assets are tested at least annually or when a triggering event occurs that could indicate a potential impairment based on indicators including progress of R&D activities, changes in projected development of assets, and changes in regulatory environment and future commercial markets. If a triggering event occurs that would indicate a potential impairment, the Company will perform a quantitative analysis to determine whether it is more likely than not that the fair value is below carrying amount. No impairment of the IPR&D asset was recognized and the mice colony model was impaired and written off during the year ended December 31, 2023.

Accrued R&D Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued R&D expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued R&D expenses include fees paid to:

- CROs in connection with performing R&D services on our behalf;
- investigative sites or other providers in connection with clinical trials;
- vendors in connection with non-clinical development activities; and
- vendors related to product manufacturing, development and distribution of clinical supplies.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple CROs that conduct and manage non-clinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed, enrollment of patients, number of sites activated and level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting expenses that are too high or too low in any particular period.

Stock-Based Compensation

We issue stock-based awards to employees and non-employees, generally in the form of stock options and restricted stock units. We account for stock-based compensation awards in accordance with FASB ASC Topic 718, Compensation—Stock Compensation ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock units and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. We measure the compensation expense of employee and non-employee services received in exchange for an award of equity instruments based on the fair value of the award on the grant date. That cost is recognized on a straight-line basis over the period during which the employee and nonemployee is required to provide service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Scholes option pricing model based on key assumptions such as expected volatility and expected term. These assumptions are primarily based on the trading price of the Company's stock, historical data, peer company data and judgment regarding future trends and factors.

We classify stock-based compensation expense in our statements of operations in the same manner in which the award recipient's payroll costs and services are classified or in which the award recipient's service payments are classified. The Company recognizes compensation expense for at least the portion of awards that are vested. Forfeitures are accounted for as they occur.

Recent Accounting Pronouncements

There were no recent accounting pronouncements that impacted the Company or are expected to have a significant effect on the consolidated financial statements.

Not Adopted as of December 31, 2023

ASU 2023-09: Income Taxes Topic 740 - Improvements to Income Tax Disclosures. This update standardizes categories for the effective tax rate reconciliation, requires disaggregation of income taxes and additional income tax-related disclosures. This update is required to be effective for the Company for fiscal periods beginning after December 15, 2024. As this accounting standard only impacts disclosures, it will not have a material impact on the Company's consolidated financial statements.

ASU 2023-07: Segment Reporting Topic 280 - Improvements to Reportable Segment Disclosures. This update requires expanded annual and interim disclosures for significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss. This update will be effective for fiscal years beginning after December 15, 2023, and is to be applied retrospectively to all periods presented in the financial statements. Early adoption is permitted. As the Company does not have segments and this accounting standard only impacts disclosures, it will not have a material impact on the Company's consolidated financial statements.

Item 7A. Quantitative and Qualitative Disclosures about Market Risks

We are exposed to market risk related to changes in interest rates. As of December 31, 2023 and 2022, we had cash, cash equivalents and investments of $407.5 million and $399.7 million, respectively. The Company's investments are primarily in U.S. Treasury Securities, Commercial Paper and Corporate and Agency Bonds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available-for-sale securities are subject to interest rate risk and will fall in value if market interest rates increase. If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at December 31, 2023, the net effect on the net fair value of our interest-sensitive marketable securities would have resulted in a hypothetical decline of $1.7 million.

Item 8. Financial Statements and Supplementary Data

The financial statements required by this Item 8 are included in Item 15 of this Annual Report.

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive and our principal financial and accounting officers, evaluated, as of the end of the period covered by this Annual Report, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of December 31, 2023, our principal executive officer and interim principal financial and accounting officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level. The term "disclosure controls and procedures," as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms.

Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and interim principal financial and accounting officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies 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 Rules 13(a)-15(f) and 15(d)-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, our principal executive and principal financial officers and effected by our Board of Directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and

- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Under the supervision and with the participation of management, including our principal executive and financial officers, we assessed our internal control over financial reporting as of December 31, 2023, based on criteria for effective internal control over financial reporting established in Internal Control — Integrated Framework (2013), issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Our management's assessment of the effectiveness of our internal control over financial reporting included testing and evaluating the design and operating effectiveness of our internal controls. In our management's opinion, we have maintained effective internal control over financial reporting as of December 31, 2023, based on criteria established in the COSO 2013 framework.

The effectiveness of our internal control over financial reporting as of December 31, 2023 has been audited by EisnerAmper LLP, an independent registered public accounting firm, as stated in their report which is included herein.

Inherent Limitations of Internal Controls

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation. 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.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

During the three months ended December 31, 2023, none of our directors or officers adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not Applicable.

PART III. — OTHER INFORMATION

Item 10. Directors, Executive Officers, and Corporate Governance

Information with respect to this item will be set forth in the Proxy Statement for the 2024 Annual Meeting of Stockholders ("Proxy Statement") under the headings "Nominees for Election as Directors," "Information about Our Executive Officers," "Information about the Board and Corporate Governance" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

Item 11. Executive Compensation

Information with respect to this item will be set forth in the Proxy Statement under the headings "Compensation Discussion and Analysis," "Executive Compensation," "Director Compensation" and "Equity Compensation Plan Information" is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

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

Information with respect to this item will be set forth in the Proxy Statement under the headings "Security Ownership of Certain Beneficial Owners and Management" and "Equity Compensation Plan Information" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

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

Information with respect to this item will be set forth in the Proxy Statement under the headings "Transactions with Related Persons" and "Information about the Board and Corporate Governance" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

Item 14. Principal Accountant Fees and Services

Our independent public accounting firm is EisnerAmper LLP, New York, New York, PCAOB Auditor ID 274.

Information with respect to this item will be set forth in the Proxy Statement under the heading "Ratification of Appointment of Independent Registered Public Accounting Firm" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

PART IV

Item 15. Exhibits, Financial Statements and Schedules

(a) The following documents are filed as part of this Annual Report:

(1) Financial Statements:

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits:

Exhibit Number	Exhibit Index Description of Exhibit
2.1	Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among Inotek Pharmaceuticals Corporation, Rocket Pharmaceuticals, Ltd. and Rome Merger Sub (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 13, 2017)
2.2	Agreement and Plan of Merger, dated September 19, 2022, by and among Rocket Pharmaceuticals, Renovacor, Inc., Zebrafish Merger Sub, Inc. and Zebrafish Merger Sub, LLC (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 20, 2022)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (incorporated by reference to Exhibit 3.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 31, 2015)
3.2	Certificate of Amendment (Reverse Stock Split) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of January 4, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.4	Certificate of Amendment to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective June 25, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2019)
3.5	Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of March 29, 2018 (incorporated by reference to Exhibit 3.2 to the Company's registration statement on Form 8-A/A, as amended (001-36829), filed with the SEC on January 11, 2018)
4.1	Form of Common Stock Certificate of Rocket Pharmaceuticals, Inc. (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
4.2	Description of Securities (incorporated by reference to Exhibit 4.8 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021)
4.3	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 15, 2023)
10.1#	Second Amended and Restated 2014 Stock Option and Incentive Plan (incorporated by reference to Appendix A to the Company's Definitive Proxy Statement (001-36829), filed with the SEC on April 30, 2018)
10.2#	Form of Incentive Stock Option Agreement (Employees) (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018)
10.3#	Form of Non-Qualified Stock Option Agreement (Employees) (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018)
10.4#	Form of Non-Qualified Stock Option Agreement (Non-Employee Directors) (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018)

Exhibit Number	Exhibit Index Description of Exhibit
10.5#	Form of Non-Qualified Stock Option Agreement (Consultants) (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018)
10.6#	Form of Restricted Stock Unit Award Agreement (incorporated by reference to Exhibit 10.6.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021)
10.7#	Rocket Pharmaceuticals, Ltd. 2015 Share Option Plan (incorporated by reference to Exhibit 10.3 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 7, 2018)
10.8**	Amended and Restated Lease Agreement, dated as of June 26, 2019, by and between the Company and Cedar Brook 12 Corporate Center, L.P. (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 8, 2019)
10.9#	Rocket Pharmaceuticals, Inc. Amended and Restated 2014 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.10 to the Company's Annual Report on Form 8-K (001-36829), filed with the SEC on March 7, 2018)
10.10#	Form of Indemnification Agreement, to be entered into between the Registrant and its directors (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
10.11#	Form of Indemnification Agreement, to be entered into between the Registrant and its officers (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
10.12#	Severance and Change in Control Policy, effective as of February 14, 2023 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 9, 2023)
10.13#*	Form of Proprietary Information, Inventions and Non-Solicitation/Non-Competition Agreement, to be entered into between the Company and its officers
10.14†	License Agreement, dated as of November 19, 2018, by and between Rocket Pharmaceuticals, Ltd. and REGENXBIO Inc. (incorporated by reference to Exhibit 10.26 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 8, 2019)
10.15	Warrant to Purchase Shares of Common Stock, dated as of December 21, 2020, by and between the Registrant and Neptune Consulting, LLC. (incorporated by reference to Exhibit 10.23 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021)
10.16	Warrant to Purchase Shares of Common Stock, dated as of December 17, 2021, by and between the Registrant and Neptune Consulting, LLC. (First Indication) (incorporated by reference to Exhibit 10.26 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on February 28, 2022)
10.17	Warrant to Purchase Shares of Common Stock, dated as of December 17, 2021, by and between the Registrant and Neptune Consulting, LLC. (Second Indication) (incorporated by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on February 28, 2022)
10.18	Registration Rights Agreement, dated as of August 27, 2021, by and among Rocket Pharmaceuticals, Inc., and each of those persons listed as an Investor on the Schedule of Inventors attached as Schedule A thereto (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on August 30, 2021)
10.19	Sales Agreement, dated February 28, 2022, by and between the Company and Cowen and Company, LLC (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on March 1, 2022)
10.20	Assignment, Assumption and Amended & Restated Warrant Agreement, dated January 16, 2023, by and among Rocket Pharmaceuticals, Inc., Zebrafish Merger Sub II, LLC, as successor to Renovacor, Inc., and Continental Stock Transfer & Trust Company (incorporated by reference to Exhibit 4.3 to the Company's Registration Statement on Form 8-A (001-36829), filed with the SEC on February 24, 2023)
10.21	Amendment No. 1 to the Sales Agreement, dated September 12, 2023, by and between the Company and Cowen and Company, LLC (incorporated by reference to Exhibit 1.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 13, 2023)
10.22	Underwriting Agreement, dated September 12, 2023, among Rocket Pharmaceuticals, Inc. and J.P. Morgan Securities LLC, Morgan Stanley & Co. LLC, Leerink Partners LLC and Cowen and Company, LLC, as representative of the several underwriters named in Schedule A thereto (incorporated by reference to Exhibit 1.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 15, 2023)
10.23#*	Rocket Pharmaceuticals, Inc. General Compensation Clawback Policy
21.1*	List of Subsidiaries
23.1*	Consent of EisnerAmper LLP
24.1*	Power of Attorney (included in the signature page)
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

Exhibit Number	Exhibit Index Description of Exhibit
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
97*	Rocket Pharmaceuticals, Inc. Nasdaq Rule 5608 Compensation Clawback Policy
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in exhibit 101)

* Filed herewith.
# Indicates management contract or compensatory plan.
† Confidential treatment has been granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
** Certain portions of this exhibit have been excluded because they are both not material and would likely cause competitive harm to the Company if publicly disclosed.
The certification attached as Exhibit 32.1 accompanying this Annual Report on Form 10- K is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Rocket Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.

Item 16. Form 10-K Summary

Not Applicable.

SIGNATURES

Pursuant to the requirements 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.

ROCKET PHARMACEUTICALS, INC.

February 27, 2024

By: /s/ Gaurav Shah, MD
Gaurav Shah, MD
Chief Executive Officer and Director
(Principal Executive Officer)

February 27, 2024

By: /s/ John Militello
John Militello
VP of Finance, Senior Controller and Treasurer
(Interim Principal Financial Officer and Principal Accounting Officer)

POWER OF ATTORNEY AND SIGNATURES

Each person whose individual signature appears below hereby constitutes and appoints Gaurav Shah, MD and John Militello, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their or his substitute or substitutes may lawfully do or cause to be done by virtue thereof.

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.

Name	Title	Date
/s/ Gaurav Shah, MD Gaurav Shah, MD	Chief Executive Officer and Director (Principal Executive Officer)	February 27, 2024
/s/ John C. Militello John C. Militello	VP, Finance, Senior Controller & Treasurer (Interim Principal Financial Officer, Principal Accounting Officer)	February 27, 2024
/s/ Carsten Boess Carsten Boess	Director	February 27, 2024
/s/ Pedro Granadillo Pedro Granadillo	Director	February 27, 2024
/s/ Gotham Makker, MD Gotham Makker, MD	Director	February 27, 2024
/s/ David P. Southwell David P. Southwell	Director	February 27, 2024
/s/ Roderick Wong, MD Roderick Wong, MD	Director	February 27, 2024
/s/ Naveen Yalamanchi, MD Naveen Yalamanchi, MD	Director	February 27, 2024
/s/ Elisabeth Björk Elisabeth Björk	Director	February 27, 2024
/s/ Fady Malik Fady Malik	Director	February 27, 2024
/s/ R. Keith Woods R. Keith Woods	Director	February 27, 2024

Rocket Pharmaceuticals, Inc.
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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of
Rocket Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Rocket Pharmaceuticals, Inc. and Subsidiaries (the "Company") as of December 31, 2023 and 2022, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2023, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the consolidated financial position of the Company as of December 31, 2023 and 2022, and the consolidated results of their operations and their cash flows for each of the years in the three-year period ended December 31, 2023, in conformity with accounting principles generally accepted in the United States of America.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the Company's internal control over financial reporting as of December 31, 2023, based on criteria established in *Internal Control - Integrated Framework* (*2013*) issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"), and our report dated February 27, 2024 expressed an unqualified opinion.

Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

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

Accruals for research and development expenses

As disclosed in Note 3 to the financial statements, the Company estimates accrued research and development expenses for existing contracts, evaluates and identifies services that have been performed for the Company, estimates the level of service performed and the associated costs incurred for the services when not yet invoiced or otherwise notified of the actual costs, and evaluates contractual milestones reached. The Company estimates costs on clinical trials in progress based on the services received and efforts expended pursuant to contracts with multiple contract research organizations (CROs), investigative sites in connection with clinical trials, and contract manufacturing organizations (CMOs). The accounts payable and accrued research and development expenses as of December 31, 2023 were approximately $13.9 million.

We identified accruals related to research and development activities as a critical audit matter due to the complexity of the estimation of those accruals related to third party CROs, CMOs and investigative sites. The complexity of the Company's estimates for these accruals was primarily the determination of progress and direct and indirect costs incurred under these arrangements, where invoicing of costs and milestones may not match the timing of services provided to date. As a result, auditor judgement was required to perform procedures and evaluate audit evidence related to the accruals for research and development expenses.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the financial statements. We obtained an understanding, evaluated the design and tested the operating effectiveness of the Company's controls over the determination of estimates of the research and development accruals, including controls over inputs used by management to make the estimates and the completeness and accuracy of the data used in the estimates. Our audit procedures also included inspection of a sample of contracts, invoices and payments, confirmation of amount owed by the Company as of December 31, 2023 for a sample of third-party research and development vendors, comparing the Company's estimates of progress to the contracts, statements of work, data confirmed by third party vendors, invoices and payments of the resulting accruals.

Impairment assessment – In-process research and development

As described in Notes 3 and 6 to the financial statements, as of December 31, 2023, the Company had an indefinite-lived intangible asset of $25.2 million which is related to in-process research and development ("IPR&D") acquired from Renovacor, Inc. in December 2022. Management reviews the IPR&D for impairment by performing a qualitative assessment of impairment indicators. If a qualitative assessment indicates that the Company's IPR&D could be impaired, a quantitative assessment is performed. If the quantitative assessment indicates the fair value of the asset is less than its carrying value, the asset is written down to its fair value. During the year ended December 31, 2023, management performed its qualitative assessment of impairment indicators for its IPR&D based on indicators including progress of research and development activities, changes in projected development of assets, and changes in regulatory environment and future commercial markets. Management made significant assumptions regarding the likelihood and extent of preclinical and clinical study timeline and related costs, probability of regulatory approval, commercialization, and sales projections.

The principal considerations for our determination that performing procedures relating to the impairment assessment of the IPR&D is a critical audit matter as there was significant judgment by management when assessing the impairment indicators. This in turn led to a high degree of auditor judgment, subjectivity and effort in performing procedures and evaluating audit evidence relating to preclinical and clinical study timeline and related costs, probability of regulatory approval, commercialization, and sales projections. In addition, the audit effort involved the use of professionals with specialized skill and knowledge to assist in performing these procedures and evaluating the audit evidence obtained from these procedures.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the financial statements. We obtained an understanding, evaluated the design and implementation and tested the operating effectiveness of controls relating to management's IPR&D impairment assessment. We evaluated the reasonableness of management's assumptions which involved consideration of whether these assumptions were consistent with (i) the Company's public announcements and filings, (ii) external market and industry data, and (iii) evidence obtained in other areas of the audit. Professionals with specialized skill and knowledge were used to assist in the evaluation of the reasonableness of the discount rate.

/s/ EisnerAmper LLP

We have served as the Company's auditor since 2016.

EISNERAMPER LLP
Iselin, New Jersey
February 27, 2024

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of
Rocket Pharmaceuticals, Inc.

Opinion on Internal Control over Financial Reporting

We have audited Rocket Pharmaceuticals, Inc. and Subsidiaries' (the "Company") internal control over financial reporting as of December 31, 2023, based on criteria established in the *Internal Control - Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2023, based on criteria established in the *Internal Control - Integrated Framework* (*2013*) issued by COSO.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the consolidated balance sheets of Rocket Pharmaceuticals, Inc.and Subsidiaries as of December 31, 2023, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2023, and the related notes and our report dated February 27, 2024 expressed an unqualified opinion.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the 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 effective internal control over financial reporting was maintained in all material respects. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

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

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

/s/ EisnerAmper LLP

EISNERAMPER LLP
Iselin, New Jersey
February 27, 2024

Rocket Pharmaceuticals, Inc.
Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 55,904	$ 140,517
Investments	317,271	215,877
Prepaid expenses and other current assets	5,047	7,666
Total current assets	378,222	364,060
Property and equipment, net	39,172	29,009
Goodwill	39,154	39,154
Intangible assets	25,150	25,724
Restricted cash	1,372	1,340
Deposits	533	608
Investments	34,320	43,276
Operating lease right-of-use assets, net	3,901	1,972
Finance lease right-of-use asset, net	44,517	46,664
Total assets	$ 566,341	$ 551,807
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 45,789	$ 36,660
Operating lease liabilities, current	925	773
Finance lease liability, current	1,791	1,736
Total current liabilities	48,505	39,169
Operating lease liabilities, non-current	2,973	1,088
Finance lease liability, non-current	19,353	19,269
Other liabilities	2,936	2,595
Total liabilities	73,767	62,121
Commitments and contingencies (Note 14)		
Stockholders' equity:		
Preferred stock, $0.01 par value, authorized 5,000,000 shares:		
Series A convertible preferred stock; 300,000 shares designated as Series A; 0 shares issued and outstanding	-	-
Series B convertible preferred stock; 300,000 shares designated as Series B; 0 shares issued and outstanding	-	-
Common stock, $0.01 par value, 120,000,000 shares authorized; 90,282,267 and 79,123,312 shares issued and 90,282,267 and 79,120,741 shares outstanding at December 31, 2023 and December 31, 2022, respectively	903	791
Treasury stock, at cost, 0 common shares at December 31, 2023 and 2,571 common shares at December 31, 2022	-	(47)
Additional paid-in capital	1,450,722	1,203,074
Accumulated other comprehensive income (loss)	319	(357)
Accumulated deficit	(959,370)	(713,775)
Total stockholders' equity	492,574	489,686
Total liabilities and stockholders' equity	$ 566,341	$ 551,807

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Operations
(in thousands, except share and per share amounts)

	For the Years Ended December 31,		
	2023	**2022**	**2021**
Revenue	$ -	$ -	$ -
Operating expenses:			
Research and development	186,342	165,570	125,476
General and administrative	73,317	58,773	41,772
Total operating expenses	259,659	224,343	167,248
Loss from operations	(259,659)	(224,343)	(167,248)
Research and development incentives	-	500	1,000
Interest expense	(1,875)	(1,862)	(2,977)
Interest and other income, net	5,288	3,889	3,068
Accretion of discount and amortization of premium on investments, net	10,651	(47)	(2,912)
Net loss	$ (245,595)	$ (221,863)	$ (169,069)
Net loss per share - basic and diluted	$ (2.92)	$ (3.26)	$ (2.67)
Weighted-average common shares outstanding - basic and diluted	84,009,004	68,148,925	63,235,417

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Comprehensive Loss
(in thousands)

	For the Years Ended December 31,		
	2023	**2022**	**2021**
Net loss	$ (245,595)	$ (221,863)	$ (169,069)
Other comprehensive loss:			
Net unrealized gain (loss) on investments	676	(196)	(119)
Total comprehensive loss	$ (244,919)	$ (222,059)	$ (169,188)

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Changes in Stockholders' Equity
(in thousands, except share amounts)

	Common Stock		Treasury	Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Stock	Capital	Income/(Loss)	Deficit	Equity
Balance at December 31, 2020	60,996,367	$ 610	$ -	$ 825,794	$ (42)	$ (322,843)	$ 503,519
Issuance of common stock pursuant to exercise of stock options	1,209,960	12	-	11,315	-	-	11,327
Issuance of common stock pursuant to conversion of notes	1,487,046	15	-	40,679	-	-	40,694
Issuance of common stock, net of issuance costs	812,516	8	-	26,346	-	-	26,354
Issuance of warrants	-	-	-	12,781	-	-	12,781
Unrealized comprehensive loss on investments	-	-	-	-	(119)	-	(119)
Stock-based compensation	-	-	-	29,237	-	-	29,237
Net loss	-	-	-	-	-	(169,069)	(169,069)
Balance at December 31, 2021	64,505,889	645	-	946,152	(161)	(491,912)	454,724
Issuance of common stock, net of issuance costs	7,820,000	78		108,060			108,138
Issuance of common stock pursuant to exercise of stock options	66,887	1	-	630	-	-	631
Issuance of common stock pursuant to vesting of restricted stock units	10,168	-	-	-	-	-	-
Issuance of common stock related to acquisition	3,420,774	34	-	70,690	-	-	70,724
Issuance of common stock related to earnout restricted stock units settlement	5,101	-	-	-	-	-	-
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	3,291,922	33	-	46,533	-	-	46,566
Treasury stock repurchase	2,571	-	(47)	-	-	-	(47)
Unrealized comprehensive loss on investments	-	-	-	-	(196)	-	(196)
Stock-based compensation	-	-	-	31,009	-	-	31,009
Net loss	-	-	-	-	-	(221,863)	(221,863)
Balance at December 31, 2022	79,123,312	791	(47)	1,203,074	(357)	(713,775)	489,686
Issuance of common stock, net of issuance costs	9,453,418	95	-	188,790	-	-	188,885
Issuance of common stock pursuant to exercise of stock options	223,145	2	-	2,229	-	-	2,231
Issuance of common stock pursuant to vesting of restricted stock units	407,999	4	-	(4)	-	-	-
Issuance of common stock pursuant to exercise of warrants	126,093	1	-	6	-	-	7
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	948,300	10	-	17,212	-	-	17,222
Unrealized comprehensive gain on investments	-	-	-	-	676	-	676
Sale of treasury stock	-	-	47	9	-	-	56
Stock-based compensation	-	-	-	39,406	-	-	39,406
Net loss	-	-	-	-	-	(245,595)	(245,595)
Balance at December 31, 2023	90,282,267	$ 903	$ -	$ 1,450,722	$ 319	$ (959,370)	$ 492,574

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	For the Years Ended December 31,		
	2023	**2022**	**2021**
Operating activities:			
Net loss	$ (245,595)	$ (221,863)	$ (169,069)
Adjustments to reconcile net loss to net cash used in operating activities:			
Accretion of discount on convertible notes	-	-	753
Depreciation and amortization of property and equipment	4,944	3,932	3,240
Amortization of finance lease right of use asset	2,154	2,334	2,133
Impairment of acquired intangible asset	574	-	-
Write down of property and equipment	311	236	261
Stock-based compensation	39,406	31,009	29,237
Accretion of discount and amortization of premium on investments, net	(10,168)	134	2,887
Expense in connection with warrant issuance	-	-	12,781
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	2,694	(3,593)	1,307
Accounts payable and accrued expenses	10,147	9,674	(4,827)
Operating lease liability and right of use asset, net	137	(120)	(11)
Finance lease liability	139	172	201
Other liabilities	341	(57)	(56)
Net cash used in operating activities	(194,916)	(178,142)	(121,163)
Investing activities:			
Purchases of investments	(390,920)	(376,327)	(245,875)
Proceeds from maturities of investments	309,326	272,894	272,443
Cash proceeds from acquisition of business, net of cash paid	-	42,726	-
Payments made to acquire right of use asset	(36)	(261)	(95)
Purchases of property and equipment	(16,436)	(8,358)	(7,620)
Net cash (used in) provided by investing activities	(98,066)	(69,326)	18,853
Financing activities:			
Issuance of common stock, net of issuance costs	188,885	108,138	26,354
Issuance of common stock, pursuant to exercise of stock options	2,231	631	11,327
Treasury stock repurchase	-	(47)	-
Issuance of common stock, pursuant to sale of treasury stock	56	-	-
Issuance of common stock, pursuant to the at-the-market offering program, net of issuance costs	17,222	46,566	-
Issuance of common stock, pursuant to exercise of warrants	7	-	-
Net cash provided by financing activities	208,401	155,288	37,681
Net change in cash, cash equivalents and restricted cash	(84,581)	(92,180)	(64,629)
Cash, cash equivalents and restricted cash at beginning of period	141,857	234,037	298,666
Cash, cash equivalents and restricted cash at end of period	$ 57,276	$ 141,857	$ 234,037
Supplemental disclosure of non-cash financing and investing activities:			
Accrued purchases of property and equipment, ending balance	$ 1,077	$ 2,095	$ 728
Operating lease liabilities	2,929	-	-
Unrealized gain (loss) on investments	676	(196)	(119)
Conversion of convertible notes into common stock	-	-	40,694
Issuance of common stock related to acquisition	-	70,724	-
Reclassification of construction in process from finance right of use asset	-	261	98
Supplemental cash flow information:			
Cash paid for interest	$ -	$ -	$ 148

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

1. Nature of Business and Basis of Presentation

Rocket Pharmaceuticals, Inc. ("Rocket" or the "Company") is a fully integrated, late-stage biotechnology company focused on the development of first, only and best in class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. The Company has three clinical-stage ex vivo lentiviral vector ("LV") programs, which include programs for:

- Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells;
- Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction; and
- Pyruvate Kinase Deficiency ("PKD"), a red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia.

In September 2023, the FDA accepted the Biologics License Application (BLA) and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 studies were completed in 2023 with regulatory filings in the United States ("U.S.") and Europe ("EU") for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., the Company also has two clinical stage and one pre-clinical stage *in vivo* adeno-associated virus ("AAV") programs, which include programs for:

- Danon disease ("DD"), a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The DD program is currently in an ongoing Phase 2 trial.
- Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), an inheritable cardiac disorder that is characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. This program received FDA clearance of an Investigational New Drug ("IND") application and the Company has initiated a Phase 1 study.
- BAG3 Dilated Cardiomyopathy ("DCM"), which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. The Company utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

The Company has global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

The Company's product candidates are in the development and clinical stage. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

The Company's consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows from operations and had an accumulated deficit of $959.4 million as of December 31, 2023. As of December 31, 2023, the Company has $407.5 million of cash, cash equivalents, and investments. Included in the $407.5 million of cash, cash equivalents and investments balance is a payable for securities that have yet to be paid of $13.1 million related to investments in securities that were purchased in 2023 and settled in 2024. The net cash balance, when adjusting for this payable would have been $394.4 million. The Company expects such resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements into 2026.

On February 28, 2022, the Company entered into a sales agreement (the "Sales Agreement"), with Cowen and Company, LLC ("Cowen"), with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock, par value $0.01 per share, having an aggregate offering price of up to $200 million (the "Shares") through Cowen as its sales agent. On September 12, 2023, the Company and Cowen entered into an amendment (the "Amended Sales Agreement") pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million. Through December 31, 2023, the Company has sold 4.2 million shares of common stock for net proceeds of $63.8 million pursuant to the at-the-market offering program (see Note 8 "Stockholders' Equity"), including 0.9 million shares for net proceeds of $17.2 million during the year ended December 31, 2023.

On October 6, 2022, the Company completed a public offering of approximately 7.8 million shares of common stock for net proceeds of $108.1 million (see Note 8 "Stockholder's Equity").

On September 15, 2023, the Company completed a public offering of approximately 9.5 million shares of our common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant. The gross proceeds from the public offering were approximately $201.3 million, net of approximately $12.4 million of offering costs, underwriting discounts and commissions, legal and other expenses for net proceeds from the offering of approximately $188.9 million (see Note 8 "Stockholder's Equity").

In the longer term, the future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

3. Summary of Significant Accounting Policies

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with accounting principles generally accepted in the U.S. ("U.S. GAAP"). All intercompany accounts have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with U.S. GAAP requires management 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 and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include but are not limited to goodwill and intangible asset impairments, the accrual of research and development ("R&D") expenses, the valuation of equity transactions, and stock-based awards. Changes in estimates and assumptions are reflected in reported results in the period in which they become known. Actual results could differ from those estimates.

Cash, Cash Equivalents and Restricted Cash

Cash, cash equivalents and restricted cash consists of bank deposits, certificates of deposit and money market accounts with financial institutions. Cash equivalents are carried at cost which approximates fair value due to their short-term nature and which the Company believes do not have a material exposure to credit risk. The Company considers all highly liquid investments with maturities of three months or less from the date of purchase to be cash equivalents. The Company's cash and cash equivalent accounts, at times, may exceed federally insured limits. The Company has not experienced any losses in such accounts.

Restricted cash consists of deposits collateralizing letters of credit issued by a bank in connection with the Company's operating leases (see Note 13 "Leases" for additional disclosures) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's corporate credit card. Cash, cash equivalents and restricted cash consist of the following:

	December 31, 2023	December 31, 2022
Cash and cash equivalents	$ 55,904	$ 140,517
Restricted cash	1,372	1,340
Total cash, cash equivalents and restricted cash	$ 57,276	$ 141,857

Government Grants

Research and development expense was presented net of reimbursements from CIRM (See Note 16 "CIRM Grant" for additional disclosure).

Concentrations of credit risk and off-balance sheet risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents and available-for-sale securities. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company's marketable securities consist of U.S. Treasury Securities, Commercial Paper and Corporate and Agency Bonds. The Company's investment policy limits the amounts the Company may invest in any one type of investment and requires all investments held by the Company to be at least AA-/Aa3 rated, thereby reducing credit risk exposure.

Investments

Investments consist of U.S. Treasury Securities and Corporate Bonds. Management determines the appropriate classification of these securities at the time they are acquired and evaluates the appropriateness of such classifications at each balance sheet date. The Company classifies its investments as available-for-sale pursuant to Financial Accounting Standards Board ("FASB") Accounting Standard Codification ("ASC") 320, Investments—Debt and Equity Securities. Investments are recorded at fair value, with unrealized gains and losses included as a component of accumulated other comprehensive income (loss) in stockholders' equity and a component of total comprehensive loss in the consolidated statements of comprehensive loss, until realized. Realized gains and losses are included in investment income on a specific-identification basis. The Company estimates expected credit losses for investments when unrealized losses exist. Unrealized losses that are credit related are recognized in the Company's consolidated statement of operations and unrealized losses that are not credit related are recognized in accumulated other comprehensive income (loss). There were no realized gains or losses on investments for the years ended December 31, 2023, 2022 and 2021. For the year ended December 31, 2023 there was net unrealized gains on investments of $0.7 million. For the years ended December 31, 2022 and 2021, there were net unrealized losses on investments of $0.2 million and $0.1 million, respectively.

Intangible Assets

Intangible assets consisted of an indefinite lived intangible IPR&D asset and a mice colony model. Intangible assets related to IPR&D projects are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time. IPR&D intangible assets which are determined to have had a decrease in their fair value are adjusted downward and an expense is recognized in R&D expenses in the Consolidated Statements of Operations. These IPR&D intangible assets are tested at least annually or when a triggering event occurs that could indicate a potential impairment based on indicators including progress of R&D activities, changes in projected development of assets, and changes in regulatory environment and future commercial markets. If a triggering event occurs that would indicate a potential impairment, the Company will perform a quantitative analysis to determine whether it is more likely than not that the fair value is below carrying amount. If a triggering event occurs that would indicate a potential impairment, the Company will perform a quantitative analysis to determine whether it is more likely than not that the fair value is below carrying amount. The annual impairment assessment for the IPR&D asset related to the Renovacor acquisition was performed as of December 1, 2023. No impairment of the IPR&D asset was recorded for the years ended December 31, 2023 and 2022. The mice colony model was impaired and written off during the year ended December 31, 2023.

Goodwill

Business combinations are accounted for under the acquisition method. The total cost of an acquisition is allocated to the underlying identifiable net assets, based on their respective estimated fair values as of the acquisition date. Determining the fair value of assets acquired and liabilities assumed requires management's judgment and often involves the use of significant estimates and assumptions, including assumptions with respect to future cash inflows and outflows, discount rates, asset lives and market multiples, among other items. Assets acquired and liabilities assumed are recorded at their estimated fair values. The excess of the purchase price over the estimated fair values of the net assets acquired is recorded as goodwill. Goodwill is tested for impairment annually as of December 31, or more frequently when events or changes in circumstances indicate that the asset might be impaired. Examples of such events or circumstances include, but are not limited to, a significant adverse change in legal or business climate, an adverse regulatory action or unanticipated competition. The Company has one segment and one reporting unit and as such review's goodwill for impairment at the consolidated level. When testing goodwill, the Company has the option to first assess qualitative factors for reporting units that carry goodwill. The qualitative assessment includes assessing the totality of relevant events and circumstances that affect the fair value or carrying value of the reporting unit. These events and circumstances include macroeconomic conditions, industry and competitive environment conditions, overall financial performance, reporting unit specific events and market considerations. The Company also considers recent valuations of the reporting unit, including the magnitude of the difference between the most recent fair value estimate and the carrying value, as well as both positive and adverse events and circumstances, and the extent to which each of the events and circumstances identified may affect the comparison of a reporting unit's fair value with its carrying value. If the qualitative assessment results in a conclusion that it is more likely than not that the fair value of a reporting unit exceeds the carrying value, then no further testing is performed for that reporting unit.

The Company performed the qualitative assessment of its goodwill and determined that it is more likely than not that the fair value of a reporting unit exceeds the carrying value of the reporting unit. As a result, the Company has determined there was no goodwill impairment as of and for the years ended December 31, 2023, 2022 and 2021.

Property and Equipment, Net

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful lives of the asset which are three to fifteen years. The Company capitalizes purchases of laboratory equipment, machinery and equipment, furniture and fixtures and leasehold improvements in relation to the facility at Cranbury, New Jersey, since it has been determined these assets have alternative future uses to the Company. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations. Costs incurred in connection with development or purchase of internal use software and cloud computing arrangements, including in-substance software licenses, are capitalized. Amortization is computed on a straight-line basis over the estimated useful life of the asset, which is six years. Capitalized software is included in property and equipment in the consolidated balance sheets.

Impairment of Long-Lived Assets

The Company reviews long-lived assets to be held and used for impairment whenever events or changes in circumstances indicate that the asset's carrying amount may not be recoverable. The Company conducted its impairment analyses of long-lived assets to be held and used in accordance with ASC 360-10-15, Impairment or Disposal of Long-Lived Assets. ASC 360-10-15 requires the Company to group assets and liabilities at the lowest level for which identifiable cash flows are largely independent of the cash flows of other assets and liabilities and evaluate the asset group against the sum of the undiscounted future cash flows. If the undiscounted cash flows do not indicate the carrying amount of the asset is recoverable, an impairment charge is measured as the amount by which the carrying amount of the asset group exceeds its fair value based on discounted cash flow analysis or appraisals. The Company recorded an impairment of the intangible long-lived asset related to the mice colony model of approximately $0.6 million during the year ended December 31, 2023. The Company also recorded write downs of property and equipment in each of the years ended December 31, 2023, 2022 and 2021.

Fair Value Measurements

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC 820, Fair Value Measurements and Disclosures, establishes a hierarchy of inputs used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair value of the Company's financial instruments, including cash and cash equivalents, restricted cash, deposits, accounts payable and accrued expenses approximate their respective carrying values due to the short-term nature of most of these instruments.

Warrants

The Company accounts for stock warrants as either equity instruments, liabilities or derivative liabilities in accordance with ASC Topic 480, Distinguishing Liabilities from Equity and/or ASC Topic 815, Derivatives and Hedging, depending on the specific terms of the warrant agreement. Liability-classified warrants are recorded at their estimated fair values at each reporting period until they are exercised, terminated, reclassified or otherwise settled. Changes in the estimated fair value of liability-classified warrants are included in interest and other income in the Company's consolidated statements of operations. Equity-classified warrants are recorded within additional paid-in capital at the time of issuance and are not subject to remeasurement.

Research and Development Expenses

R&D expenses, which include salaries and staff costs, license costs, manufacturing and development costs, clinical trial expenses, depreciation and amortization expenses, regulatory and scientific consulting fees, as well as contract research, and stock-based compensation expense, are accounted for in accordance with ASC Topic 730, Research and Development. Accordingly, R&D costs are expensed as incurred.

Foreign Currency Transactions

Certain transactions during the years ended December 31, 2023, 2022 and 2021 are primarily denominated in Euros and British pounds. Gains and losses on foreign currency transactions were not significant for the years ended December 31, 2023, 2022 and 2021.

Treasury Stock

The Company records treasury stock at cost.

Stock-Based Compensation

The Company issues stock-based awards to employees and non-employees, generally in the form of stock options and restricted stock units ("RSUs"). The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718, Compensation - Stock Compensation ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock units and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. The Company measures the compensation expense of employee and non-employee services received in exchange for an award of equity instruments based on the fair value of the award on the grant date. That cost is recognized on a straight-line basis over the period during which the employee and nonemployee is required to provide service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Scholes option pricing model based on key assumptions such as expected volatility and expected term. The Company's estimates of these assumptions are primarily based on the trading price of the Company's stock, historical data, peer company data and judgment regarding future trends and factors.

The Company classifies stock-based compensation expense in its consolidated statements of operations in the same manner in which the award recipient's payroll costs and services are classified or in which the award recipient's service payments are classified. The Company recognizes compensation expense for at least the portion of awards that are vested. Forfeitures are accounted for as they occur.

New York State Life Sciences Research and Development Tax Credit

New York State ("NYS") allows investors and owners of emerging technology companies focused on biotechnology to claim a tax credit against their NYS Tax return for certain expenditures incurred in NYS, including applicable R&D related expenditures. The credit is recognized as R&D incentives when the eligibility and amount has been approved by NYS. During the years ended December 31, 2023, 2022 and 2021, the Company recorded R&D incentive income of $0, $0.5 million, and $1.0 million, respectively related to the NYS Life Sciences Research and Development Tax Credit.

Income Taxes

The Company accounts for income taxes under the asset and liability method. The Company recognizes deferred tax assets and liabilities for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases, as well as for operating loss and tax credit carryforwards. The Company measures deferred tax assets and liabilities using enacted tax rates expected to apply to taxable income in the years in which the Company expects to recover or settle those temporary differences. The Company recognizes the effect of a change in tax rates on deferred tax assets and liabilities in the results of operations in the period that includes the enactment date. The Company reduces the measurement of a deferred tax asset, if necessary, by a valuation allowance if it is more likely than not that the Company will not realize some or all of the deferred tax asset.

The Company's deferred tax assets relate primarily to its net operating loss carryforwards and other balance sheet differences. In accordance with ASC 740, Income Taxes, the Company recorded a full valuation allowance to fully offset the net deferred tax asset because it is not more likely than not that the Company will realize future benefits associated with these deferred tax assets at December 31, 2023 and 2022.

The Company accounts for uncertainty in income taxes recognized in the financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Net Loss Per Share

The Company calculates net loss per share in accordance with FASB ASC 260, Earnings per Share. Basic net loss per share attributable to common shareholders is computed by dividing the net loss attributable to common shareholders by the weighted average number of common shares outstanding for the period. Warrants that meet the definition of equity classification and that requires the holder to pay little or no consideration to receive shares upon exercise are considered outstanding in the context of basic earnings per share. Diluted net loss attributable to common shareholders is computed by adjusting net loss attributable to common shareholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share attributable to common shareholders is computed by dividing the diluted net loss attributable to common shareholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares. For purposes of this calculation, outstanding options are considered potential dilutive common shares.

Segment Reporting

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment.

Comprehensive Loss

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions, and other events and circumstances from non-owner sources and consists of net loss and changes in unrealized gains and losses on investments.

Leases

The Company determines if an arrangement is a lease at inception. Operating and finance leases are presented in the Company's consolidated balance sheet as right-of-use assets from leases, current lease liabilities and long-term lease liabilities. Certain of the Company's lease agreements contain renewal options; however, the Company does not recognize right-of-use assets or lease liabilities for renewal periods unless it is determined that the Company is reasonably certain of renewing the lease at inception or when a triggering event occurs. As the Company's leases do not provide an implicit rate, the Company estimated the incremental borrowing rate in calculating the present value of the lease payments using an estimate of the Company's collateralized borrowing rate for debt with a similar term. The Company has utilized its incremental borrowing rate based on the long-term borrowing costs of comparable companies in the biotechnology industry. Since the Company elected to account for each lease component and its associated non-lease components as a single combined lease component, all contract consideration was allocated to the combined lease component. Some of the Company's lease agreements contain rent escalation clauses (including index-based escalations). For operating leases, the Company recognizes the minimum rental expense on a straight-line basis based on the fixed components of a lease arrangement. The Company will amortize this expense over the term of the lease beginning with the lease commencement date. Variable lease components represent amounts that are not fixed in nature and are not tied to an index or rate and are recognized as incurred.

Recent Accounting Pronouncements

There were no recent accounting pronouncements that impacted the Company or are expected to have a significant effect on the consolidated financial statements.

Accounting Pronouncements Not Adopted as of December 31, 2023

ASU 2023-09: Income Taxes Topic 740 - Improvements to Income Tax Disclosures. This update standardizes categories for the effective tax rate reconciliation, requires disaggregation of income taxes and additional income tax-related disclosures. This update is required to be effective for the Company for fiscal periods beginning after December 15, 2024. As this accounting standard only impacts disclosures, it will not have a material impact on the Company's Consolidated Financial Statements.

ASU 2023-07: Segment Reporting Topic 280 - Improvements to Reportable Segment Disclosures. This update requires expanded annual and interim disclosures for significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss. This update will be effective for fiscal years beginning after December 15, 2023, and is to be applied retrospectively to all periods presented in the financial statements. Early adoption is permitted. As the Company does not have segments and this accounting standard only impacts disclosures, it will not have a material impact on the Company's consolidated financial statements.

4. Fair Value of Financial Instruments

Items measured at fair value on a recurring basis are the Company's investments. The following table sets forth the Company's financial instruments that were measured at fair value on a recurring basis by level within the fair value hierarchy:

	Fair Value Measurements as of December 31, 2023 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents:				
Money market mutual funds	$ 50,737	$ -	$ -	$ 50,737
U.S. Treasury Securities	-	2,487	-	2,487
	50,737	2,487	-	53,224
Investments:				
U.S. Treasury Securities	-	312,696	-	312,696
Corporate Bonds	-	38,895	-	38,895
	-	351,591	-	351,591
Total assets	$ 50,737	$ 354,078	$ -	$ 404,815
Liabilities:				
Warrant liability	$ -	$ -	$ 1,876	$ 1,876
Total liabilities	$ -	$ -	$ 1,876	$ 1,876

	Fair Value Measurements as of December 31, 2022 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 90,527	$ -	$ -	$ 90,527
Commercial Paper	-	3,899	-	3,899
U.S. Treasury Securities	-	3,848	-	3,848
Corporate Bonds	-	8,618	-	8,618
	90,527	16,365	-	106,892
Investments:				
Commercial Paper	-	1,151	-	1,151
U.S. Treasury Securities	-	189,444	-	189,444
Corporate Bonds	-	60,905	-	60,905
Agency Bonds	-	7,653	-	7,653
	-	259,153	-	259,153
Total assets	$ 90,527	$ 275,518	$ -	$ 366,045
Liabilities:				
Warrant liability	$ -	$ -	$ 1,512	$ 1,512
Total liabilities	$ -	$ -	$ 1,512	$ 1,512

The Company classifies its money market mutual funds as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices in active markets without any valuation adjustment. The Company classifies its U.S. Treasury Securities, Commercial Paper and Corporate and Agency Bonds as Level 2 assets as these assets are not traded in an active market and have been valued through a third-party pricing service based on quoted prices for similar assets.

The reconciliation of the Company's warrant liability, which is recorded as part of Other Liabilities in the consolidated balance sheets, measured at fair value on a recurring basis using unobservable inputs (Level 3) is as follows:

	Warrant Liability
Balance, December 31, 2021	$ -
Acquisition of Renovacor	1,512
Balance, December 31, 2022	$ 1,512
Fair value adjustments	364
Balance, December 31, 2023	$ 1,876

The Company utilizes a Black-Scholes model to value the Private Warrants (see Note 10 "Warrants") at each reporting period, with changes in fair value recognized in the consolidated statements of operations. The estimated fair value of the warrant liability is determined using Level 3 inputs. Inherent in an options pricing model are assumptions related to expected share-price volatility, expected life, risk-free interest rate and dividend yield. The Company estimates the expected volatility of its common stock based on historical volatility of a peer group, considering the expected remaining life of the Private Warrants. The risk-free interest rate is based on the U.S. Treasury zero-coupon yield curve on the valuation date for a maturity similar to the expected remaining life of the Private Warrants. The expected life of the Private Warrants is assumed to be equivalent to their remaining contractual term. The dividend rate is based on the historical rate, which the Company anticipates will remain at zero.

The fair value of the Private Warrants has been estimated with the following assumptions:

	December 31, 2023	December 1, 2022
Stock price	$ 29.50	$ 18.39
Exercise price	$ 65.23	$ 65.23
Expected volatility	68.83%	71.25%
Risk-free interest rate	4.70%	4.14%
Expected dividend yield	-	-
Expected life (years)	1.31	2.39
Fair value per warrant	$ 3.04	$ 2.45

The fair value change from December 1, 2022, to December 31, 2022, was immaterial.

5. Property and Equipment, Net

The Company's property and equipment consisted of the following:

	December 31, 2023	December 31, 2022
Laboratory equipment	$ 29,232	$ 21,905
Machinery and equipment	12,325	11,326
Computer equipment	244	244
Furniture and fixtures	2,777	2,135
Leasehold improvements	6,723	589
Internal use software	1,903	1,903
	53,204	38,102
Less: accumulated depreciation and amortization	(14,032)	(9,093)
Total property, plant and equipment, net	$ 39,172	$ 29,009

Depreciation and amortization during the years ended December 31, 2023, 2022, and 2021 was $4.9 million, $3.9 million and $3.2 million, respectively.

6. Intangible Assets and Goodwill

The Company's intangible assets consisted of an indefinite lived intangible IPR&D asset and a mice colony model received from the acquisition of Renovacor. Intangible assets as of December 31, 2023 and 2022 are summarized as follows:

December 31, 2023	Gross Carrying Value	Accumulated Amortization	Intangible Assets, Net
In process research & development	$ 25,150	$ -	$ 25,150
Total intangible assets	$ 25,150	$ -	$ 25,150

December 31, 2022	Gross Carrying Value	Accumulated Amortization	Intangible Assets, Net
In process research & development	$ 25,150	$ -	$ 25,150
Mice colony model	574	-	574
Total intangible assets	$ 25,724	$ -	$ 25,724

The Company holds intangible assets as a result of the acquisition of Renovacor (see Note 17 "Renovacor Acquisition"). The decrease in gross carrying value of intangibles at December 31, 2023 compared to December 31, 2022 was due to an impairment charge related to a reduction in the estimated fair value of the mice colony model to reflect the limited benefit of the model.

The carrying value of Goodwill as of December 31, 2023 and 2022 was $39.2 million and included $8.3 million as a result of the acquisition of Renovacor in 2022 (see Note 17 "Renovacor Acquisition"):

	Carrying Value
Balance, December 31, 2021	$ 30,815
Acquisition of Renovacor	8,339
Balance, December 31, 2022 and 2023	$ 39,154

7. Accounts Payable and Accrued Expenses

The Company's accounts payable and accrued expenses consisted of the following:

	December 31, 2023	December 31, 2022
Research and development	$ 13,867	$ 19,100
Investment payable	13,137	-
Employee compensation	9,930	10,006
Property and equipment	1,077	2,095
Professional fees	6,006	1,436
Acquisition related expenses	-	1,153
Government grant payable	-	597
Other	1,772	2,273
Total accounts payable and accrued expenses	$ 45,789	$ 36,660

The $13.1 million investment payable was related to investment purchases of available-for-sale securities in 2023 that settled in 2024.

8. Stockholders' Equity

Common Stock

The Company is currently authorized to issue up to 120,000,000 shares of $0.01 par value common stock. All issued shares of common stock are entitled to vote on a 1 share/1 vote basis.

Second Amended and Restated 2014 Stock Option and Incentive Plan

In March 2018, Rocket's Board of Directors approved the Second Amended and Restated 2014 Stock Option and Incentive Plan (the "Revised 2014 Plan") which was approved by the Company's shareholders at the Annual Meeting held on June 25, 2018.

Treasury Stock

During the year ended December 31, 2023, the Company recorded sale of treasury stock of $0.06 million. During the year ended December 31, 2022, the Company recorded a repurchase of treasury stock of $0.05 million for shares withheld to pay the payroll tax liability of the vesting of RSUs. There was no treasury stock as of December 31, 2023.

At-the-Market Offering Program

On February 28, 2022, the Company entered into the Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares through Cowen as its sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to the Company's shelf registration statement on Form S-3. The Company filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. The Company will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. The Company has provided Cowen with customary indemnification and contribution rights. The Company reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through December 31, 2023, the Company sold 4.2 million shares under the at-the-market offering program for gross proceeds of $65.8 million, less commissions of $2.0 million for net proceeds of $63.8 million. During the year ended December 31, 2023, the Company sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commissions of approximately $0.6 million for net proceeds of $17.2 million. During the year ended December 31, 2022, the Company sold 3.3 million shares under the at-the-market offering program for gross proceeds of $48.0 million, less commissions of $1.4 million for net proceeds of $46.6 million. On September 12, 2023, the Company and Cowen entered into the Amended Sales Agreement pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million.

Public Offerings

On October 6, 2022, the Company completed a public offering of approximately 7.8 million shares of our common stock at a public offering price of $14.75 per share. The gross proceeds from the public offering were approximately $115.3 million, net of $7.2 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $108.1 million.

On September 15, 2023, the Company completed a public offering of approximately 9.5 million shares of its common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant. The gross proceeds from the public offering were approximately $201.3 million, net of $12.4 million of offering costs, underwriting discounts and commissions, legal and other expenses for net proceeds from the offering of $188.9 million.

9. Stock-Based Awards

Stock Option Valuation

The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the stock options granted to employees, non-employees and directors were as follows:

	For the Years Ended December 31,		
	2023	2022	2021
Risk-free interest rate	4.00%	2.19%	0.83%
Expected term (in years)	5.82	5.09	5.84
Expected volatility	73.32%	64.12%	69.27%
Expected dividend yield	0.00%	0.00%	0.00%
Exercise price	$ 20.24	$ 18.56	$ 51.20
Fair value of common stock	$ 20.24	$ 18.56	$ 51.20

The following table summarizes stock option activity for the years ended December 31, 2023 and 2022:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Yrs)	Aggregate Intrinsic Value
Outstanding as of December 31, 2021	11,143,761	$ 14.51	5.95	$ 128,817
Conversion of Renovacor awards	367,852	4.63	0.40	
Granted	2,305,910	13.94	5.53	
Exercised	(66,887)	9.43		514
Cancelled or forfeited	(611,766)	32.55		
Outstanding as of December 31, 2022	13,138,870	$ 14.52	5.46	$ 118,767
Granted	2,370,862	20.24	8.76	
Exercised	(223,145)	10.00		2,318
Cancelled or forfeited	(422,591)	29.82		
Outstanding as of December 31, 2023	14,863,996	$ 15.07	5.16	$ 250,602
Options vested and exercisable as of December 31, 2023	11,657,122	$ 13.57	4.13	$ 217,871
Options unvested as of December 31, 2023	3,206,874	$ 20.50	8.91	$ 32,731

The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2023, 2022 and 2021 was $13.37, $9.88, and $31.07, respectively.

The total fair value of options vested during the years ended December 31, 2023, 2022 and 2021 was $26.5 million, $34.9 million and $22.6 million, respectively.

Restricted Stock Units ("RSU")

The following table summarizes the RSU activity for the years ended December 31, 2023 and 2022:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2021	23,500	$ 30.61
Conversion of Renovacor awards	28,798	0.49
Granted	1,047,301	15.91
Vested	(38,966)	23.15
Forfeited	(67,759)	15.94
Unvested as of December 31, 2022	992,874	$ 16.49
Granted	1,018,322	19.67
Vested [(1)]	(408,119)	16.61
Forfeited	(112,720)	17.92
Unvested as of December 31, 2023	1,490,357	$ 18.53

(1) Common stock issued is net of 120 shares related to taxes.

The total fair value of RSU's vested during the years ended December 31, 2023, 2022 and 2021 was $6.8 million, $0.8 million, and $0.4 million, respectively.

Stock-Based Compensation

Stock-based compensation expense recognized by award type is as follows:

	For the Years Ended December 31,		
	2023	2022	2021
Stock options	$ 29,091	$ 27,620	$ 28,811
Restricted stock units	10,315	3,389	426
Total stock-based compensation expense	$ 39,406	$ 31,009	$ 29,237

Stock-based compensation expense by classification included within the consolidated statements of operations and comprehensive loss was as follows:

	For the Years Ended December 31,		
	2023	2022	2021
Research and development	$ 17,509	$ 12,466	$ 11,954
General and administrative	21,897	18,543	17,283
Total stock-based compensation expense	$ 39,406	$ 31,009	$ 29,237

As of December 31, 2023, the Company had an aggregate of $52.5 million of unrecognized stock-based compensation expense, which is expected to be recognized over the weighted average period of 1.83 years.

10. Warrants

A summary of the Company's outstanding warrants at December 31, 2023 is as follows:

Exercise Price	Outstanding	Grant/Assumption Date	Expiration Date
$57.11	603,386	December 21, 2020	December 21, 2030
33.63	301,291	August 9, 2021	August 9, 2031
22.51	153,155	December 17, 2021	December 17, 2031
22.51	153,155	December 17, 2021	December 17, 2031
65.23	617,050	December 1, 2022	April 23, 2025
65.23	760,086	December 1, 2022	December 1, 2026
$0.01	3,126,955	September 15, 2023	N/A
Total	5,715,078		

The following table below is the summary of changes in the Company's warrants to purchase common stock for the years ended December 31, 2023 and 2022:

	Number of Warrant Shares Outstanding and Exercisable	Exercise Price per Share
Balance as of December 31, 2021	1,218,038	
Assumed Renovacor Private warrants - liability	617,050	$ 65.23
Assumed Renovacor Public warrants - equity	760,086	65.23
Assumed Renovacor Pre-funded warrants - equity	126,093	0.06
Balance as of December 31, 2022	2,721,267	
Issued	3,126,955	0.01
Exercised	(126,093)	0.06
Expired	(7,051)	$ 24.42
Balance as of December 31, 2023	5,715,078	

The Company issued warrants to a related party during the years ended December 31, 2023 and 2021. For the year ended December 31, 2023, the Company sold pre-funded warrants to purchase 3,126,955 shares of common stock (see Note 8 "Stockholders Equity"). For the year ended December 31, 2021, the Company incurred a non-cash R&D expense of $12.8 million related to the issuance of the 2021 warrants (see Note 18 "Related Party Transactions"). No warrants were issued to the related party during the year ended December 31, 2022.

Assumed Renovacor Public Warrants

In conjunction with the Renovacor acquisition (see Note 17 "Renovacor Acquisition") Rocket assumed outstanding original 8,622,644 pre-acquisition public warrants ("Public Warrants") which were issued in connection with Renovacor's initial public offering in April 2020. Each Public Warrant initially entitled the holder to purchase one-half of one share of Renovacor's common stock at an initial exercise price of $11.50 per whole share, subject to adjustment. No fractional shares will be issued upon exercise of the Public Warrants.

Therefore, the Public Warrants must be exercised in multiples of two Public Warrants for one share of the Company's common stock. The Public Warrants will expire five years following the SPAC merger closing date, or earlier upon redemption or liquidation. As a result of the acquisition, these former Renovacor Public Warrants were converted into Rocket warrants with a right to purchase 760,086 of Rocket common shares at an exercise price of $65.23 per share.

The Company may redeem the Public Warrants in whole and not in part at a price of $0.01 per Public Warrant at any time during the exercise period upon a minimum of 30 days' prior written notice of redemption if, and only if, the last sale price of the Company's common stock equals or exceeds $90.75 per share for any 10 trading days within a 30-trading day period ending on the third business day prior to the date on which the Company sends the notice of redemption to the warrant holders; and if, and only if, there is a current registration statement in effect with respect to the shares of common stock underlying such Public Warrants at the time of redemption and for the entire 30-day trading period referred to above and continuing each day thereafter until the date of redemption.

To date, certain of the above conditions have not been met to redeem the Public Warrants. If the Company calls the Public Warrants for redemption, management will have the option to require all holders that wish to exercise the Public Warrants to do so on a "cashless basis," as described in the warrant agreement. The exercise price and number of shares of Common Stock issuable upon exercise of the Public Warrants may be adjusted in certain circumstances including in the event of a stock dividend, or recapitalization, reorganization, merger or consolidation. Additionally, in no event will the Company be required to net cash settle the Public Warrants.

The Company determined that the Public Warrants met all of the criteria for equity classification. Accordingly, upon closing of the Merger in 2022, the Public Warrants were recorded as a component of additional paid-in capital of $3.4 million.

Assumed Renovacor Private Warrants

Prior to the acquisition, Renovacor had outstanding 3,500,000 warrants ("Private Warrants") which were issued simultaneously with the closing of the Renovacor Initial Public Offering ("Renovacor IPO"), pursuant to a private placement transaction. Each Private Warrant was exercisable to purchase one share of Renovacor's common stock at an exercise price of $11.50. The Private Warrants are identical to the Public Warrants except that the Private Warrants (i) will be exercisable for cash (even if a registration statement covering the shares of common stock issuable upon exercise of such warrants is not effective) or on a cashless basis, at the holder's option, and (ii) will not be non-redeemable by the Company, in each case, so long as they are held by the initial purchasers or their permitted transferees. If the Private Warrants are held by someone other than the initial purchasers or their permitted transferees, the Private Warrants will be redeemable by the Company and exercisable by such holders on the same basis as the Public Warrants. The Private Warrants purchased by Chardan Capital Markets will not be exercisable more than five years from the effective date of the Renovacor IPO, in accordance with FINRA Rule 5110(f)(2)(G)(i), as long as Chardan Capital Markets or any of its related persons beneficially own these Private Warrants.

As a result of the acquisition, the Private Warrants were converted into warrants with a right to purchase 617,050 of Rocket common shares at an exercise price of $65.23 per share.

The Company determined that the Private Warrants did not meet all of the criteria for equity classification. Accordingly, the Company classifies the Private Warrants as a derivative liability in other liabilities in the consolidated balance sheets. The Company measures the fair value of the warrants at the end of each reporting period and recognizes changes in the fair value from the prior period in the Company's operating results for the current period. See Note 4 "Fair Value of Financial Instruments" for discussion of fair value measurement of the warrant liability.

Assumed Renovacor Pre-Funded Warrants

Concurrently with the execution of the SPAC Merger Agreement, Renovacor entered into subscription agreements (the "Subscription Agreements"), with certain investors ("PIPE Investors"), including Chardan Healthcare, certain stockholders of Old Renovacor and certain other institutional and accredited investors, pursuant to which, on the SPAC Closing Date, and concurrently with the closing of the SPAC Business Combination, the PIPE Investors purchased an aggregate of 2,284,776 shares of Renovacor's common stock, at a price of $10.00 per share, and a pre-funded warrant entitling the holder thereof to purchase 715,224 shares of Renovacor's common stock (the "Pre- Funded Warrant") at an initial purchase price of $9.99 per share underlying the Pre-Funded Warrant, for aggregate gross proceeds of approximately $30.0 million (the "PIPE Investment"). The Pre-Funded Warrant was immediately exercisable at an exercise price of $0.01 and is exercisable indefinitely, provided that the holder of the Pre-Funded Warrant was prohibited from exercising such Pre-Funded Warrant in an amount that would cause such holder's beneficial ownership of our Common Stock to exceed 9.99%, which limitation may be increased up to 19.99% at the option of the holder from time to time.

As a result of the acquisition, these Pre-Funded Warrants were converted into warrants with a right to purchase 126,093 of Rocket common shares at an exercise price of $0.06 per share. These warrants were exercised in January 2023.

The Company determined that the Pre-Funded Warrants met all of the criteria for equity classification. Accordingly, upon closing of the Merger, the Public Warrants were recorded as a component of additional paid-in capital of $2.3 million.

RTW Pre-Funded Warrants

In September 2023, in connection with the Company's public offering, the Company sold approximately 3.1 million Pre-Funded Warrants to purchase shares of the Company's common stock to funds affiliated with RTW Investments, LP, the Company's largest shareholder. The Pre-Funded Warrants were sold for a price of $15.99 per share underlying the Pre-Funded Warrant and were immediately and indefinitely exercisable at an exercise price of $0.01.

11. Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows:

	For the Years Ended December 31,		
	2023	2022	2021
Numerator:			
Net loss attributable to common stockholders	$ (245,595)	$ (221,863)	$ (169,069)
Denominator:			
Weighted-average common shares outstanding - basic and diluted	84,009,004	68,148,925	63,235,417
Net loss per share attributable to common stockholders - basic and diluted	$ (2.92)	$ (3.26)	$ (2.67)

In 2023, the Company included the 3,126,955 potential shares from the pre-funded warrants acquired by RTW as it was determined that these met the definition for equity classification and only requires the holder to pay $0.01 per share upon exercise.

The Company excluded the following potential shares of common stock, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	For the Years Ended December 31,		
	2023	2022	2021
Warrants exercisable for common shares	2,588,123	2,721,267	1,218,038
Restricted stock units convertible for common shares	1,490,357	992,874	23,500
Options to purchase common shares	14,863,996	13,138,870	11,143,761
	18,942,476	16,853,011	12,385,299

12. Income Taxes

No provision for federal or state income taxes was recorded during the years ended December 31, 2023, 2022 and 2021, as the Company incurred operating losses and maintains a full valuation allowance against its net deferred tax assets.

A reconciliation of income tax benefit computed at the statutory federal income tax rate to income taxes as reflected in the financial statements is as follows:

	For the Years Ended December 31,		
	2023	2022	2021
U.S. federal tax at statutory rate	21.0%	21.0%	21.0%
Foreign rate differential	(17.3%)	(16.9%)	(13.0%)
Change in state tax apportionment	1.4%	(0.1%)	0.1%
Stock compensation	0.1%	0.1%	1.5%
Return to provision	1.7%	—%	—%
Transfer pricing adjustments	—%	—%	(22.8%)
Valuation allowance	(7.0)%	6.3%	4.6%
Federal NOL true-up	0.3%	(2.7%)	—%
Tax credits	0.0%	(6.7%)	8.7%
Other	(0.2%)	(1.0%)	(0.1%)
Effective tax rate	—%	—%	—%

On October 4, 2023, the Governor of Massachusetts signed into law a bill that included the adoption of a single sales apportionment factor effective on January 1, 2025. As required under ASC 740, the Company has accounted for the deferred tax impacts of this tax law change in the period the tax law was enacted, which has the impact of reducing its state deferred tax assets. The impact of the tax law change is offset by a change in valuation allowance.

Intellectual property rights in different jurisdictions are reflected in the Company's effective tax rate.

The significant components of the Company's deferred income tax assets and liabilities after applying the enacted corporate tax rates are as follows:

	As of December 31,		
	2023	2022	2021
Deferred income tax assets (liabilities)			
R&D credits	$ 20,984	$ 20,984	$ 35,766
Net operating losses and credit carryforwards	45,313	33,718	26,789
Capitalized research and development costs	17,205	19,085	19,753
Stock-based compensation	28,499	19,781	11,552
Warrants	9,283	8,390	8,382
Intangible assets	(5,787)	(5,424)	—
Other	(8,086)	(6,332)	(8,881)
Valuation allowance	(108,472)	(91,263)	(93,361)
Net deferred income tax asset (liability)	$ (1,061)	$ (1,061)	$ -

As of December 31, 2023, the Company had federal and state net operating loss ("NOL") carryforwards of approximately $197.7 million and $77.2 million, respectively. The state NOL begins to expire in 2026. Additionally, $197.7 million of the federal NOL can be carried forward indefinitely. The Company has federal R&D credits of $21.0 million which will begin to expire in 2038.

As required by ASC 740, Income Taxes, the Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which are comprised principally of NOL carryforwards and capitalized research and development costs. As a result of the fact that the Company has incurred tax losses from inception, management has determined that it is more likely than not that the Company will not recognize the benefits of federal and state net deferred tax assets and, as a result, a full valuation allowance has been established against its net deferred tax assets as of December 31, 2023, 2022 and 2021. The Company has offset certain deferred tax liabilities with deferred tax assets that are expected to generate offsetting deductions within the same period. During the years ended December 31, 2023 and 2022, the valuation allowance increased by $17.2 million and decreased by $2.1 million, respectively. Realization of deferred tax assets is dependent upon the generation of future taxable income.

Under Internal Revenue Code Section 382, if a corporation undergoes an "ownership change," the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income may be limited. The Company has completed a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since the Company became a "loss corporation" as defined in Section 382. The Company experienced multiple ownership changes occurring in 2005, 2007, 2015, and 2018. The ownership change has and will continue to subject our pre-ownership change net operating loss carryforwards to an annual limitation, which will significantly restrict our ability to use them to offset taxable income in periods following the ownership change. In general, the annual use limitation equals the aggregate value of our stock at the time of the ownership change multiplied by a specified tax-exempt interest rate. As a result of the ownership change, the Company is limited to an approximate $1.7 million annual limitation on our ability to utilize our pre-merger NOL's and R&D Credits. Due to this limitation, approximately $91.2 million of the $127.1 million pre-merger Federal NOL will expire unutilized as the cumulative limitation amount over a 20-year carryforward period is $35.8 million. Additionally, $4.9 million of federal R&D credits will expire unutilized. As a result, the Company has reduced its deferred tax assets related to the Federal NOL and federal R&D credits by an aggregate of $4.9 million which is offset by the corresponding decrease in the valuation allowance. In conjunction with the Renovacor Acquisition (see Note 17 "Renovacor Acquisition"), the Company acquired Renovacor's federal NOL's of $46.4 million. The Company has completed a study to assess whether an ownership change has occurred as a result of the transaction. The Company experienced ownership changes occurring in 2021 and 2022. As a result of the ownership changes, the Company is limited on our ability to utilize our Renovacor NOL's. As a result of the Company's study, any limitation under IRC Sec. 382 would not result in NOLs expiring unused.

The Company evaluated intercompany transfer pricing agreements. Based on a review of the 2018, 2019 and 2020 tax years, the Company determined that a markup of 10% should have been applied to R&D expenses paid for on behalf of Rocket Pharmaceuticals, Ltd by Rocket Pharmaceuticals, Inc. The net impact was to reduce the Company's net operating losses for 2018, 2019 and 2020. No income tax expense was recorded as a result of these adjustments.

The calculation of tax liabilities involves dealing with uncertainties in the application of complex tax laws and regulations for both federal taxes and the many states in which the Company operates or does business in. ASC 740 states that a tax benefit from an uncertain tax position may be recognized when it is more likely than not that the position will be sustained upon examination, including resolutions of any related appeals or litigation processes, on the basis of the technical merits.

The Company records uncertain tax positions as liabilities in accordance with ASC 740 and adjusts these liabilities when our judgment changes as a result of the evaluation of new information not previously available. Because of the complexity of some of these uncertainties, the ultimate resolution may result in a payment that is materially different from the Company's current estimate of the unrecognized tax benefit liabilities. These differences will be reflected as increases or decreases to income tax expense in the period in which new information is available. As of December 31, 2023, 2022 and 2021, the Company has not recorded any uncertain tax positions in its financial statements.

The Company recognizes interest and penalties related to unrecognized tax benefits on the income tax expense line in the accompanying consolidated statements of operations. As of December 31, 2023 and 2022, no accrued interest or penalties are included on the related tax liability line in the consolidated balance sheets.

13. Leases

Finance Lease

The Company has a lease for a facility in Cranbury, New Jersey, consisting of 103,720 square feet of space including areas for offices, process development, research and development laboratories and 50,000 square feet dedicated to AAV Current Good Manufacturing Practice ("cGMP") manufacturing facilities to support the Company's pipeline (such lease, as amended, the "NJ Lease Agreement"). The NJ Lease Agreement has a 15-year term from September 1, 2019, with an option to renew for two consecutive five-year renewal terms.

Estimated rent payments for the NJ Lease Agreement are $1.2 million per annum, payable in monthly installments, depending upon the nature of the leased space, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately $29.3 million over the 15-year term of the lease. The Company paid a cash security deposit of $0.3 million to the landlord in connection with the NJ Lease Agreement which has been reflected as part of deposits in the consolidated balance sheets as of December 31, 2023 and 2022.

Operating Leases

On June 7, 2018, the Company entered into a three-year lease agreement for office space in the Empire State Building in New York, NY (the "ESB Lease Agreement"). In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit (the "Empire LOC") for $0.9 million. On March 26, 2021, the Company entered into Amendment No. 1 to the ESB Lease Agreement ("ESB Lease Amendment") that extended the term of the lease agreement to June 30, 2024, reduced the rent payments going forward, and reduced the Empire LOC to $0.8 million. The Empire LOC serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires August 29, 2024.

The Company has a certificate of deposit of $0.8 million with a bank as collateral for the Empire LOC which is classified as part of restricted cash in the consolidated balance sheets as of December 31, 2023, and 2022, respectively.

On January 4, 2018, in connection with the Reverse Merger with Inotek, the Company assumed an operating lease for Inotek's former headquarters in Lexington, Massachusetts, with a term which ended on February 28, 2023. In July 2018, the Company signed an agreement to sublease a portion of the Lexington, Massachusetts space and in September 2018, the Company signed an agreement to sublease the remaining portion of the Lexington, Massachusetts space. Rental income received under the sublease agreement totaled $0.3 million for the year ended December 31, 2023, and $0.4 million for the years ended December 31, 2022 and 2021. These amounts were netted against rent expense in the consolidated statements of operations. A security deposit of $0.2 million was returned to the Company in April 2023.

On November 15, 2022, the Company entered into a lease agreement with a lease term until October 31, 2024, for laboratory space in Madrid, Spain. The lease commenced on April 1, 2023 and the Company recognized a right-of-use asset and corresponding lease liability of approximately $0.2 million each.

On December 1, 2022, in connection with the Renovacor acquisition (see Note 17 "Renovacor Acquisition"), the Company acquired the Renovacor operating leases for space at facilities in Hopewell, New Jersey and Cambridge, Massachusetts with remaining lease terms of approximately 10.25 and 1.3 years, respectively. As of December 31, 2023, lease commencement dates have occurred for all leases and the Company recognized total right-of-use assets of $3.8 million with corresponding total lease liabilities of $3.6 million at lease commencement dates, which include right-of-use assets of $2.7 million and lease liabilities of $2.6 million for leases that commenced in 2023. The Company intends to sublease the facilities in Hopewell, New Jersey and signed an agreement to sublease one of these facilities in January 2024.

Rent expense was $2.3 million, $1.2 million, and $1.1 million for the years ended December 31, 2023, 2022 and 2021, respectively.

The total restricted cash balance for the Company's operating and finance leases as of December 31, 2023 and 2022 was $0.8 million.

Operating lease cost was $1.4 million, $0.8 million, and $0.6 million for the years ended December 31, 2023, 2022 and 2021, respectively.

The following table summarizes lease cost for the years ended December 31, 2023, 2022 and 2021:

	For the Years Ended December 31,		
Lease cost	**2023**	**2022**	**2021**
Operating lease cost	$ 1,377	$ 818	$ 645
Finance lease cost:			
Amortization of right of use assets	2,154	2,139	2,140
Interest on lease liabilities	1,875	1,861	1,845
Total lease cost	$ 5,406	$ 4,818	$ 4,630

The following table summarizes the maturity of the Company’s lease liabilities on an undiscounted cash flow basis:

Fiscal Year Ending December 31,	**December 31, 2023**
2024	$ 931
2025	567
2026	571
2027	506
2028	522
Thereafter	2,419
Total lease payments	$ 5,516
Less: interest	(1,618)
Total operating lease liabilities	$ 3,898

Fiscal Year Ending December 31,	**December 31, 2023**
2024	$ 1,791
2025	1,856
2026	1,912
2027	1,969
2028	2,028
Thereafter	41,003
Total lease payments	$ 50,559
Less: interest	(29,415)
Total finance lease liability	$ 21,144

The following table summarizes the operating and financing lease liabilities and right-of-use assets as of December 31, 2023 and 2022:

	December 31, 2023	**December 31, 2022**
Operating right-of-use assets	$ 3,901	$ 1,972
Operating current lease liabilities	$ 925	$ 773
Operating noncurrent lease liabilities	2,973	1,088
Total operating lease liabilities	$ 3,898	$ 1,861
Finance right-of-use assets	$ 44,517	$ 46,664
Finance current lease liability	$ 1,791	$ 1,736
Finance noncurrent lease liability	19,353	19,269
Total finance lease liability	$ 21,144	$ 21,005

Other information	For the Years Ended December 31, 2023	2022	2021
Cash paid for amounts included in the measurement of lease liabilities:			
Operating cash flows from operating leases	$ 1,229	$ 938	$ 655
Cash flows from finance lease	$ 1,736	$ 1,689	$ 1,644
Weighted-average remaining lease term - operating leases	8.0 years	4.8 years	2.5 years
Weighted-average remaining lease term - finance lease	20.7 years	21.7 years	22.7 years
Weighted-average discount rate - operating leases	8.32%	6.44%	4.46%
Weighted-average discount rate - finance lease	8.96%	8.69%	8.96%

14. Commitments and Contingencies

Litigation

From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities. Although the results of litigation and claims cannot be predicted with certainty, the Company does not believe it is party to any other claim or litigation the outcome of which, if determined adversely to the Company, would individually or in the aggregate be reasonably expected to have a material adverse effect on its business. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors.

Indemnification Arrangements

Pursuant to its bylaws and as permitted under Delaware law, the Company has indemnification obligations to directors, officers, employees or agents of the Company or anyone serving in these capacities. The maximum potential amount of future payments the Company could be required to pay is unlimited. The Company has insurance that reduces its monetary exposure and would enable it to recover a portion of any future amounts paid. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

Throughout the normal course of business, the Company has agreements with vendors that provide goods and services required by the Company to run its business. In some instances, vendor agreements include language that requires the Company to indemnify the vendor from certain damages caused by the Company's use of the vendor's goods and/or services. The Company has insurance that would allow it to recover a portion of any future amounts that could arise from these indemnifications. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

15. Agreements Related to Intellectual Property

The Company, directly and through its subsidiary Spacecraft Seven, LLC, has various license and research and collaboration arrangements. The transactions principally resulted in the acquisition of rights to intellectual property which is in the preclinical phase and has not been tested for safety or feasibility. In all cases, the Company did not acquire tangible assets, processes, protocols or operating systems. The Company expenses the acquired intellectual property rights as of the acquisition date on the basis that the cost of intangible assets purchased from others for use in research and development activities, has no alternative future uses.

License Agreements with CIEMAT

In March 2016, the Company entered into a license agreement with CIEMAT, CIBER, and FIISFJD, (collectively, "CIEMAT"), granting Rocket worldwide, exclusive rights to certain patents, know-how and other intellectual property relating to LVs containing the human PKLR gene solely within the field of treating PKD. Under the terms of the agreement, the Company is obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public, (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, Rocket is obligated to pay CIEMAT an up-front payment, royalty payments based on net sales of products or processes involving any of the licensed intellectual property, developmental and regulatory milestone payments, and sublicense revenue payments. The Company is responsible for prosecuting and maintaining the licensed patents at its expense, in cooperation with CIEMAT. Rocket also has the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with CIEMAT. For five years following the effective date of the license agreement, the Company has a right of first refusal to license any improvements to the licensed intellectual property obtained by CIEMAT at market value. Rocket is obligated to license (without charge) to CIEMAT for non-commercial use any improvements to the licensed intellectual property that it creates.

As consideration for the licensed rights, Rocket paid CIEMAT an initial upfront license fee of €0.03 million (approximately $0.03 million) which was expensed as R&D costs. The Company is obligated to make aggregate milestone payments of up to €1.4 million (approximately $1.5 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the PKD license, Rocket is obligated to pay a low to mid-single digit percentage royalty on net sales, subject to specified adjustments, by the Company or its sublicensees or affiliates. In the event that Rocket enters into a sublicense agreement with a sublicensee, it will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

Rocket may terminate this agreement at any time by providing CIEMAT with 90 days advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

In July 2016, Rocket entered into a license agreement with CIEMAT granting it worldwide, exclusive rights to certain patents, know-how, data and other intellectual property relating to LVs containing the FANCA gene solely within the field of human therapeutic uses of VSV-G packaged integration component LVs for FA type-A gene therapy. This license is only sublicensable with the prior consent of CIEMAT, not to be unreasonably withheld. Under the terms of the agreement, Rocket is obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, the Company is obligated to pay CIEMAT an up-front payment, royalty payments based on net sales of products or processes involving any of the licensed intellectual property, regulatory and financing milestone payments, and sublicense revenue payments. The Company is responsible for prosecuting and maintaining the licensed patents at our expense, in cooperation with CIEMAT. Rocket also has the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with CIEMAT. For five years following the effective date of the license agreement, the Company has a right of first refusal to license any improvements to the licensed intellectual property obtained by CIEMAT at market value. Rocket is obligated to license (without charge) to CIEMAT for non-commercial use any improvements to the licensed intellectual property that it creates.

As consideration for the licensed rights, Rocket paid CIEMAT an initial upfront license fee of €0.1 million (approximately $0.1 million), which was expensed as R&D costs. The Company is obligated to make aggregate milestone payments of up to €5.0 million (approximately $6.0 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the license, Rocket is obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by Rocket or its sublicensees or affiliates. In the event that the Company enters into a sublicense agreement with a sublicensee, the Company will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

Rocket may terminate this agreement at any time by providing CIEMAT with 90 days' advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

License Agreement for LAD-I with CIEMAT and UCLB

The Company entered into a license agreement in November 2017, effective September 2017, with CIEMAT and UCL Business PLC ("UCLB"), collectively referred to as ("Licensors"), granting the Company worldwide, exclusive rights to certain patents, know-how and other intellectual property relating to LVs containing the human LAD-I gene solely within the field of treating LAD-I. Under the terms of the agreement, Rocket is obligated to use commercially reasonable efforts to (a) develop and obtain regulatory approval for one or more products or processes covered by the licensed intellectual property, introduce such products or processes into the commercial market and then make them reasonably available to the public, (b) develop or commercialize at least one product or process covered by the licensed intellectual property in at least one country for at least two uninterrupted years following regulatory approval, and (c) use the licensed intellectual property in an adequate, ethical and legitimate manner. In exchange for the license, the Company is obligated to pay Licensors an up-front payment, royalty payments in the mid-single digit percentages based on net sales of products or processes involving any of the licensed intellectual property, developmental and regulatory milestone payments, and sublicense revenue payments. Rocket is responsible for prosecuting and maintaining the licensed patents at its expense, in cooperation with Licensors. The Company also has the first responsibility to enforce and defend the licensed patents against infringement and/or challenge, in cooperation with Licensors. For five years following the effective date of the license agreement, Rocket has a right of first refusal to license any improvements to the licensed intellectual property obtained by Licensors at market value. The Company is obligated to license (without charge) to Licensors for non-commercial use any improvements to the licensed intellectual property that it creates.

As consideration for the licensed rights, Rocket paid Licensors an initial upfront license fee of €0.03 million (approximately $0.04 million), which was expensed as R&D costs. The Company is obligated to make aggregate payments of up to €1.4 million (approximately $1.5 million) to Licensors upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the LAD-I license, Rocket is obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by the Company or its sublicensees or affiliates. In the event that the Company enters into a sublicense agreement with a sublicensee, it will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

Rocket may terminate this agreement at any time by providing the Licensors with 90 days advance notice. The license is in effect for a duration for each of the countries defined in this agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

License Agreement for DD with UCSD

In February 2017, the Company entered into a License Agreement with The Regents of the University of California, represented by its San Diego campus ("UCSD"), under which UCSD granted us an exclusive, sublicensable, worldwide license to certain intellectual property rights for the treatment of lysosomal storage diseases, including DD. In exchange for the license, the Company became obligated to make an up-front payment, certain clinical and commercial milestone payments, royalty payments (on net sales of products covered by a valid claim within the licensed intellectual property), maintenance fees and sublicense revenue payments. The upfront license fee of $0.05 million was expensed as research and development costs in 2020. The Company is obligated to make aggregate milestone payments of up to $1.5 million to UCSD upon the achievement of specified development and regulatory milestones for the treatment of DD. A reduced schedule of milestone payments applies to achieving the same milestones for additional indications. With respect to any commercialized products covered by the agreement, the Company is obligated to pay a low single digit percentage royalty on net sales, subject to specified adjustments. If it enters into a sublicense agreement with a sublicensee, it will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances. The Company is also subject to certain diligence milestones for development of a product using the intellectual property licensed from UCSD under this agreement. The term of the license agreement with UCSD is through the expiration of the licensed patents, some of which are still in the pending application phase.

REGENXBIO, Inc. License

On November 19, 2018, the Company entered into a license agreement with REGENXBIO Inc. ("RGNX"), pursuant to which the Company obtained an exclusive license for all U.S. patents and patent applications related to RGNX's NAV AAV-9 vector for the treatment of DD in humans by *in vivo* gene therapy using AAV-9 to deliver any known LAMP2 transgene isoforms and all possible combinations of LAMP2 transgene isoforms (the "Field"), as well as an exclusive option to license (the "Option Right") all U.S. patents and patent applications for two additional NAV AAV vectors in the Field (each, a "Licensed Patent" and collectively, the "Licensed Patents").

Under the terms of the license agreement, the Company is obligated to use commercially reasonable efforts to develop, commercialize, market, promote and sell products incorporating the Licensed Patents ("Licensed Products"). Unless the license agreement is terminated earlier as provided below, the license from RGNX expires on a country-by-country, Licensed Product-by-Licensed Product basis until the later of the expiration date of the last to expire of the last valid claim of the applicable Licensed Patent or ten years after the first commercial sale of a Licensed Product in such country. The license agreement provides that RGNX may terminate the agreement upon a material breach by the Company if the Company does not cure such breach within a specified notice period if the Company commences a challenge against RGNX or certain of its licensors to declare or render invalid or unenforceable the licensed patents or upon the Company's bankruptcy or insolvency. The Company may terminate the agreement in its entirety or terminate one or more of the licensed vectors at any time upon six months' notice. The Company's Option Right expired four years from the date of the license agreement.

In consideration for the rights granted to the Company under the license agreement, the Company made an upfront payment to RGNX of $7.0 million. The license agreement provides for royalties payable to RGNX in the high-single digits to low-teens on net sales levels of Licensed Products during the royalty term. If successful, the Company will be required to make milestone payments to RGNX of up to $13.0 million for each Licensed Product upon the achievement of specified clinical development and regulatory milestones in the U.S. and EU. In addition, the Company shall pay RGNX 20% of the payment fees received from a priority review voucher issued in connection with or otherwise related to a Licensed Product. These royalty obligations are subject to specified reductions if additional licenses from third parties are required. The Company must also pay RGNX a portion of all non-royalty sublicense income (if any) received from sublicensees. The Company paid and expensed a $1.0 million license fee payment under the RGNX agreement upon the dosing of the first DD patient in 2019 and a $2.0 million license fee payment upon initiation of a Phase 2 pivotal trial in 2023. There were no additional milestones achieved or related payments made during the years ended December 31, 2023, 2022 and 2021.

16. CIRM Grants

LAD-I CIRM Grant

On April 30, 2019, the CIRM awarded the Company up to $7.5 million under a CLIN2 grant award to support the clinical development of its LV-based gene therapy for RP-L201. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase 1/2 patients enrolled at the U.S. clinical site, UCLA Mattel Children's Hospital, led by principal investigator Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. Through December 31, 2023, the Company has received $5.8 million in total RP-L201 grants from CIRM. As of December 31, 2023, the Company met the final CIRM milestone and recorded a receivable of $0.05 million, included in prepaid and other current assets in the consolidated balance sheet, recorded as a reduction of research and development expenses. The Company received the $0.05 million final milestone payment on January 2, 2024.

17. Renovacor Acquisition

On September 19, 2022, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Renovacor, a Delaware corporation pursuant to which, on December 1, 2022, the Company acquired Renovacor (the "Renovacor Acquisition"). On December 1, 2022, pursuant to the terms of the Merger Agreement, (i) Merger Sub I merged with and into the Company (the "First Merger") and (ii) the Company, as the surviving company of the First Merger merged with and into Merger Sub II (the "Second Merger"), with Merger Sub II surviving the Second Merger. Subject to the terms and conditions of the Merger Agreement, at the closing of the Renovacor Acquisition each share of Renovacor's common stock outstanding immediately prior to the effective time of the First Merger were canceled and converted into the right to receive 0.1763 (the "Exchange Ratio") of fully paid and non-assessable shares of the Company common stock, which was determined on the basis of the exchange formula set forth in the Merger Agreement that was subject to adjustment depending on the level of the Renovacor's net cash at the closing. Prior to the market opening on December 1, 2022, Renovacor shares ceased to trade on NYSE and upon the closing of the acquisition, Renovacor's outstanding common stock were converted into 3,391,976 shares of Rocket common stock.

Total consideration for the Renovacor Acquisition was $72.3 million, consisting of $62.4 million for common stock outstanding, $2.7 million for the portion of equity compensation attributable to the pre-combination service period, and $7.2 million for assumed warrants. The consideration was based on the estimated fair values on the acquisition date of (i) 3,391,976 common shares issued for shares outstanding for common shares of Renovacor, (ii) estimated fair value of employee stock options to acquire 367,852 common shares of the Company, (iii) 28,798 common shares issued for employee time-vesting RSUs, and (iv) warrants to acquire 1,503,229 common shares (see Note 10 "Warrants").

The total consideration for the acquisition of Renovacor of $72.3 million consisted of the following:

	Shares	Value	Total
Stock consideration	3,391,976	$ 18.39	$ 62,378
Cash consideration[(1)]			29
Stock options	367,852		2,163
Time-vesting RSUs	28,798		512
Assumed warrants[(2)]	1,503,229		7,183
Total consideration	5,291,855		$ 72,265

(1) Represents consideration paid for cash in lieu of fractional shares.
(2) Assumed Renovacor Warrants of $7,183 with $5,671 classified as equity and $1,512 classified as liabilities.

The acquisition has been accounted for as a business combination using the acquisition method of accounting which requires that assets acquired and liabilities assumed be recognized at their fair values as of the acquisition date and that the fair value of acquired IPR&D assets are classified as indefinite-life assets until the successful completion or abandonment of the associated research and development efforts.

The purchase price allocation resulted in the following amounts being allocated to the assets acquired and liabilities assumed at the acquisition date based on their respective fair values summarized below:

Working capital[(1)]	$	(5,210)
Cash and cash equivalents		42,755
Property and equipment		1,414
Operating lease right-of-use assets		1,161
Other non-current assets		113
IPR&D		25,150
Other intangible asset		574
Operating lease liability		(970)
Deferred tax liability		(1,061)
Net assets acquired		63,926
Goodwill		8,339
Purchase consideration	$	72,265

(1) Includes other receivables, prepaid expenses, account payable and accrued liabilities

The fair value assigned to acquired IPR&D was based on the present value of expected after-tax cash flows attributable to Renovacor's most advanced AAV-based gene therapy targeting BAG3-DCM. The present value of expected after-tax cash flows was determined by estimating the after-tax costs to complete development into a commercially viable product, estimating future revenue and ongoing expenses to produce, and discounting the resulting net cash flows to present value. The cost and revenue projections used were reduced based on the assessed probabilities of different stages of development. Acquired IPR&D will be accounted for as an indefinite-lived intangible asset until regulatory approval in a major market or discontinuation of development.

The excess of purchase price over the fair value of amounts assigned to identifiable assets acquired and liabilities assumed represents the goodwill amount of $8.3 million resulting from the acquisition. The goodwill recorded as part of the acquisition is primarily attributable to the broadening of the Company's portfolio and research capabilities, deferred taxes and the assembled workforce. The goodwill attributable to the acquisition has been recorded as a non-current asset in the Company's consolidated balance sheet and is not amortized, but subject to review for impairment annually. The Company incurred $3.2 million of acquisition related general and administrative costs during the year ended December 31, 2022.

18. Related Party Transactions

On December 21, 2020, the Company entered into a consulting agreement with a related party. Pursuant to the consulting agreement, the related party provides certain business development and asset identification consulting services to the Company. On August 9, 2021, the Company issued a warrant exercisable for 301,291 shares of common stock to the same related party for business development and asset identification consulting services ("August 2021 Warrant"). The Company recorded a non-cash R&D expense of $7.6 million during year ended December 31, 2021, related to the issuance of the August 2021 warrant. On December 17, 2021, the Company issued warrants exercisable for 153,155 and 153,155 shares of common stock, respectively to the same related party for business development and asset identification consulting services ("December 2021 Warrants"). The Company recorded a non-cash R&D expense of $5.2 million during year ended December 31, 2021, related to the issuance of the December 2021 warrant. Total non-cash R&D expense of $12.8 million during the year ended December 31, 2021, related to the issuance of the August 2021 and December 2021 warrants.

In September 2021, the Company entered into a consulting agreement with a member of the Board of Directors for pipeline development, new asset evaluation, and corporate strategy. In lieu of cash for services to be provided under the consulting agreement during its one-year term, the Company granted the board member options to purchase 20,000 shares of the Company's common stock with a fair value of $0.4 million.

In June 2023, the Company entered into a consulting agreement with the spouse of one of the Company's executive officers for information technology advisory services. The Company incurred expenses of approximately $0.02 million for the year ended December 31, 2023, relating to services provided under this agreement.

In September 2023, in connection with the Company's public offering, the Company sold approximately 3.1 million pre-funded warrants to purchase shares of the Company's common stock to funds affiliated with RTW Investments, LP, the Company's largest shareholder (see Note 8 "Stockholders' Equity").

19. 401(k) Savings Plan

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the years ended December 31, 2023, 2022, and 2021 was $1.4 million, $0.9 million, and $0.6 million, respectively.