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, 2021
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	**04-3475813**
(State or Other Jurisdiction of Incorporation or Organization)	**(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 if any, every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

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

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

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

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

The aggregate market value of the common stock held by non-affiliates of the registrant as of June 30, 2021 (the last business day of the registrant's most recently completed second fiscal quarter) was approximately $1.9 billion, based upon the closing price on the Nasdaq Global Market reported for such date.

As of February 22, 2022, there were 64,505,889 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 2022 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 never 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 "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "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:

- 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 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 establish key collaborations and vendor relationships for our product candidates and any other future 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;
- unanticipated delays due to manufacturing difficulties, including the development of our direct manufacturing capabilities for our AAV programs, and any supply constraints or changes in the regulatory environment; 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;
- uncertainties associated with obtaining and enforcing 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;
- natural and manmade disasters, including pandemics such as COVID-19, and other force majeures, which could impact our operations, and those of our partners and other participants in the health care industry, and which could adversely impact our clinical studies, preclinical research activities, and drug supply;
- 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 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:

- The outbreak of the novel strain of coronavirus, SARS-CoV-2, which causes COVID-19, has and may continue to adversely impact our business, including our preclinical and clinical studies.
- 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 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.
- Initial or interim 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.
- 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 or the European Union, or other jurisdictions, which would limit our ability to realize our full market potential.
- 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.
- 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 no experience in manufacturing, and there can be no assurance that we will be able to successfully manufacture products.
- 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.
- Even if approved, we may not successfully commercialize our product candidates.
- We may not be successful in our efforts to expand a 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.
- 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 United States, in particular the European Union, a variety of risks associated with international operations could harm our business.
- If we are unable to obtain and maintain adequate patent protection for products and related technology, our ability to successfully commercialize our products may be harmed.
- If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.
- We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights and we may be unable to protect our rights to, or use, our technology.
- If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed.
- If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our markets.
- RTW Investments, LP, our largest stockholder, may have the ability to significantly influence matters submitted to stockholders for approval.

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 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 clinical-stage, multi-platform 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 ("LVV") programs. These 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 rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., we have a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. Additional discovery efforts on a gene therapy program for the less common FA subtypes C and G is ongoing. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program is to be returned to academic innovators. Although we believe that gene therapy may be beneficial to patients afflicted with this disorder, we have opted to focus available resources towards advancement of RP-A501, RP-L102, RP-L201 and RP-L301, based on the compelling clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

Gene Therapy Overview

Genes are composed of sequences of deoxyribonucleic acid ("DNA"), which 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, LVV and AAV. We believe that our LVV and AAV-based programs have the potential to offer a significant therapeutic benefit to patients that is durable (long-lasting).

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.

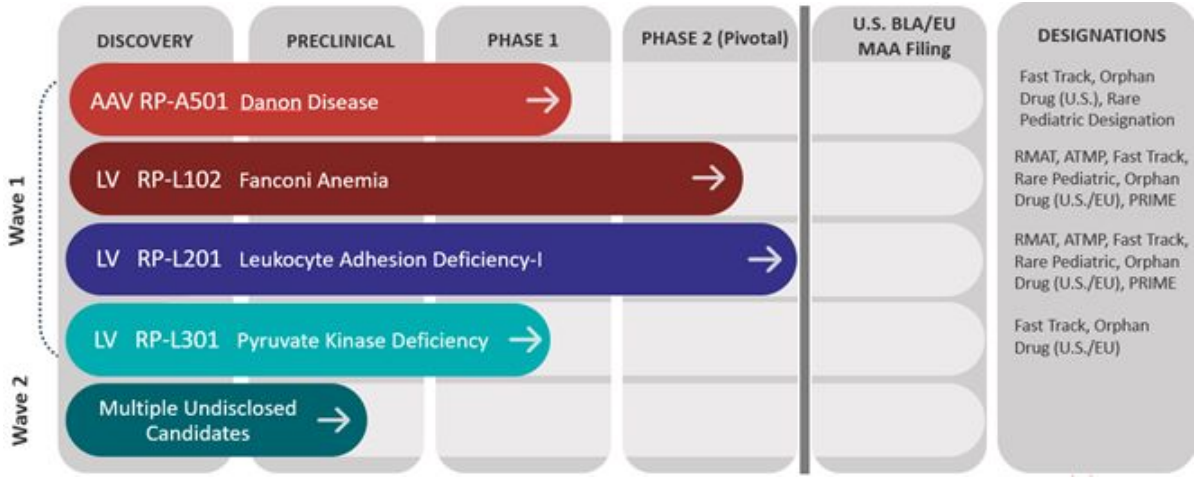
Essential Terminology.

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

Term	Definition	Optimal Ranges
	LVV 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 LVV 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 Rocket's programs and product candidates for both LVV and AAV programs:



Descriptions of these conditions and the Rocket programs for each is set forth below.

LVV Programs. Rocket's LVV-based programs utilize third-generation, self-inactivating lentiviral vectors to target selected rare diseases. Currently, Rocket is developing LVV programs to treat FA, LAD-I, and PKD.

AAV Program.

Overview of Danon Disease

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, liver disease, 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 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 Danon disease, which is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the EU.

Rationale for Gene Therapy in Danon Disease

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

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

Data disclosed from our Phase 1 study of RP-A501 in November 2021 and January 2022 included safety and clinical activity results from the three patients treated with the low dose of 6.7e13 vg/kg and from two patients treated with the higher dose of 1.1e14 vg/kg, and early safety information from the initial pediatric patient (pediatric cohort is age 8-14 years) treated with the low dose of 6.7e13 vg/kg.

Efficacy assessments include evaluation of New York Heart Association (NYHA) Functional Classification, which 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. Brain natriuretic peptide (BNP) is a blood-based evaluation and a key marker of heart failure with prognostic significance in CHF and cardiomyopathies. Other efficacy parameters include echocardiographic measurements of heart thickness, most notably the thickness of the left ventricular posterior wall (LVPW), and importantly, measurement of LAMP2B gene expression both via immunohistochemistry and Western blot, as obtained via endomyocardial biopsy. Biopsied heart tissue is also evaluated on electron microscopy for 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.

In November 2021 and January 2022, data for the ongoing Phase 1 trial of RP-A501 was presented, including efficacy parameters for the low and high dose cohorts in patients aged 15 and older with at least 12 months follow-up (n=5). An improvement in NYHA Class (from II to I) was observed in three patients (two low-dose and one high-dose) who had closely monitored immunosuppression with follow-up greater than one year and stabilization was observed in one low-dose patient without a closely monitored immunosuppressive regimen. A substantial improvement in BNP, a key marker of heart failure, was observed in all three low-dose patients and one high-dose patient. Among the three low-dose patients, BNP decreased from a pretreatment baseline by 57% at 24 months, 79% at 18 months, and 75% at 15 months, respectively. In one high-dose patient, BNP decreased from a pretreatment baseline by 67% at 12 months. In patients with closely monitored immunosuppression (two low-dose and one high-dose) left ventricular (LV) posterior wall thickness improved (average 23% decrease compared to pretreatment baseline) and ejection fraction improved or stabilized (average 20% increase compared to pretreatment baseline) at 12 to 18 months on echocardiography. Severe and progressive wall thickening is a hallmark of the hypertrophic cardiomyopathy of Danon Disease and is a major contributor to early mortality in male patients. Cardiac output remained normal for all patients with improved or stable left heart filling pressures as measured by cardiac catheterization. Three low-dose patients and one high-dose patient demonstrated improvements in the 6-minute walk test (6MWT). One low-dose patient improved from a pretreatment baseline of 443 meters (m) to 467 m at 24 months. The second low-dose patient improved from a pretreatment baseline of 405 m to 410 m at 18 months. The third low-dose patient improved from a pretreatment baseline of 427 m to 435 m at 15 months. One high-dose patient improved from a pretreatment baseline of 436 m to 492 m at 12 months. Evidence of sustained cardiac LAMP2B gene expression by immunohistochemistry and Western blot with qualitative improvement of vacuoles and cardiac tissue architecture on electron microscopy was observed at both dose levels. Sustained cardiac LAMP2B gene expression by immunohistochemistry was observed in all three patients with a closely monitored immunosuppressive regimen. Specifically, LAMP2B gene expression by immunohistochemistry in the low-dose (6.7e13 vg/kg) was 68% in one patient at Month 12 and 92% in another patient at Month 9. In one patient who received the high-dose (1.1e14 vg/kg), LAMP2B gene expression by immunohistochemistry was 100% at Month 12.

One of the patients receiving therapy on the high dose cohort had progressive heart failure and underwent a heart transplant at Month 5 following therapy. This patient had more advanced disease than the 4 other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished LV ejection fraction (35%) on echocardiogram and markedly elevated LV filling pressure prior to treatment. His clinical course was characteristic of DD progression. Assessments regarding gene transduction from the explanted heart are summarized below:

Explanted Heart

- Analysis of the explanted heart revealed significant fibrosis consistent with advanced DD.
- Myocardial tissue from the explanted heart at 5 months post-treatment displayed 100% LAMP2B protein expression by immunohistochemistry throughout non-fibrotic cardiac regions including the ventricles and other essential targeted areas.

RP-A501 was generally well tolerated at the 6.7e13 vg/kg dose level, or lower dose. All observed adverse effects were reversible with no lasting sequelae. Early transaminase and creatinine kinase elevations returned to baseline or decreased. No unexpected and serious drug product-related adverse events or severe adverse events were observed in this low dose cohort. The most common adverse events were predominantly mild, not associated with clinical symptoms and were related to elevated transaminases post-treatment. Elevation in transaminases and creatinine kinases was observed in all three low-dose patients and returned to baseline levels within the first one to two months post-treatment. There was also a transient and reversible decline in platelets observed in these three patients. These changes were largely responsive to corticosteroids and other immunosuppressive therapies. All patients were given oral steroids to prevent or minimize potential immune-related events. Corticosteroids were associated with transient exacerbation of DD-associated skeletal myopathy, which resolved upon discontinuation of steroid therapy. At the higher dose administered (1.1e14 vg/kg), additional immunosuppressive therapies were stipulated and administered to mitigate the immune response associated with RP-A501. As disclosed in December 2020, one of the two patients receiving the 1.1e14 vg/kg dose had more advanced heart failure than the others, and was the heaviest patients treated to-date (receiving the highest absolute AAV9 dose). This patient experienced a non-persistent, immune-related event that was classified as a drug product-related serious adverse event. This thrombotic microangiopathy (TMA) event (which was later reclassified as a Sudden Unexpected Serious Adverse Reaction (SUSAR)) was believed to be likely due to immune-mediated complement activation, resulting in reversible thrombocytopenia and acute kidney injury requiring eculizumab and transient hemodialysis. This patient regained normal kidney function within three weeks. (This event occurred in the same patient in whom RP-A501 was not associated with clinical stabilization or improvement, and who required a heart transplant 5 months post-therapy).

Following transplant, this patient has been clinically stable and reports resolution of a baseline skeletal myopathy that was present prior to treatment. Analysis of the explanted heart is described above. Of note, this patient had more advanced heart failure at time of treatment; the clinical protocol has been modified to exclude enrollment of DD with end-stage CHF/cardiomyopathy. In May 2021, 5 months after details of this event were disclosed and after recognition of complement-mediated TMA in other systemic AAV programs, the FDA placed the study on clinical hold. In response to the FDA's clinical hold, we amended the trial protocol in order to enable more defined mechanisms for prevention, early recognition and management of complement-mediated adverse events. The FDA lifted the clinical hold on August 16, 2021 and dosing of the pediatric cohort was initiated in the fourth quarter of 2021.

Based on the activity observed in the low dose cohort and to mitigate complement-mediated TMA (safety concerns observed in the high dose cohort) and in agreement with the FDA, we are focusing on the low dose (6.7e13 vg/kg) and we will no longer administer doses of 1.1e14 vg/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 immunosuppressive 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 therapy. As announced in January 2022, the initial pediatric patient received RP-A501 therapy (6.7e13 vg/kg dose level) without evidence of significant complement activation and with stable platelet levels; there was no worsening of the patient's baseline DD-related skeletal myopathy during the weeks following RP-A501.

Fanconi Anemia Complementation Group A (FANCA):

Fanconi Anemia Overview

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 more frequent hematologic correction of the disorder, HSCT is associated with both acute and long-term risks, including transplant-related mortality, graft versus host disease ("GVHD"), 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 during the early years of life. We believe that the development of a broadly applicable autologous gene therapy can be transformative for these patients.

Current Therapy

Allogeneic HSCT may be curative for the hematologic manifestations of FA and is currently considered a standard-of-care in FA. However, HSCT is limited in that not all patients have a suitable donor and there is associated short term mortality and potential for acute and chronic GVHD with HSCT, especially in patients who do not receive an allograft from a sibling-human leukocyte antigen ("HLA")-matched donor. 100-day mortality following allogeneic HSCT continues to be in the 10 to 15% range due to infection, graft failure and other complications. In a European Group for Blood and Marrow Transplant 2013 publication, a retrospective analysis detailed results from 795 FA patients receiving HSCT from 1972 to 2010 in which Grade 2-4 Acute GVHD was reported in 19 to 36% of patients and Chronic GVHD was identified in 16 to 20% of patients.

HSCT likely increases the already high risk of subsequent solid tumor malignancies for patients with FA, most notably squamous carcinoma of the head and neck ("SCCHN"). Based on the findings in one series of data, HSCT was associated with a 4-fold increase in SCCHN risk relative to FA patients who did not receive a transplant, with cancers developing at an earlier age. In another more recent but unpublished series, patients with HSCT who developed GVHD had a 30+-fold increase in SCCHN risk.

Other therapies utilized for FA include androgens, corticosteroids, and hematopoietic growth factors, although the benefits of these therapies are considered modest and transient for the majority of patients. Side effects may also be considerable. For androgens, for example, these include masculinization, short stature, hepatitis, liver adenomas and hepatocellular carcinoma.

Because of the severity of the disease and limitations with existing standards-of-care, additional, minimally-toxic therapies are urgently needed in FA, especially if these can be administered with reduced short- and long-term toxicity relative to allogeneic HSCT.

Rationale for Gene Therapy in FA

Gene therapy has been considered a compelling investigative therapeutic option in FA since the genetic basis of the disorder was characterized and has been the subject of studies in both preclinical models and in several clinical studies. In addition to the monogenic nature of each patient's disease. We believe there are several critical factors that will help lead our gene therapy programs into the next generation of promising therapy:

- *The ability of HSCT to cure the hematologic component of FA is proof-of-principle that gene therapy will work in FA.* If a sufficient number of hematopoietic stem cells ("HSCs") with a correct (non-FA) gene are able to engraft in the bone marrow of an FA patient, the blood component of FA can be eradicated, including both the risk of bone marrow failure and of leukemia. We believe that gene therapy with a patient's own gene-corrected blood stem cells will work in a similar manner, but likely with fewer side effects than those resulting from an allogeneic transplant and with reduced long-term treatment cost burden.
- *Mosaicism in FA patients: this is a condition in which a second mutation enables formation of a functional FA protein and leads to stabilization or correction of blood counts, in some cases enabling decades of bone-marrow-failure free survival.* Mosaicism occurs because gene-corrected FA stem and progenitor cells have a selective advantage over uncorrected FA cells; this phenomenon has demonstrated that a modest number of gene corrected HSCs can repopulate a patient's blood and bone marrow with corrected (non-FA) cells. A comprehensive review of all known cases of somatic mosaicism has demonstrated a correlation with lowered risk of both bone marrow failure and hematologic malignancy. This selective advantage also has been demonstrated by the results of our initial FANCOLEN-I gene therapy study in Madrid, Spain, in which patients received gene-corrected cells without any chemotherapy conditioning; the percentage of blood and bone marrow cells containing the corrected FA gene has increased progressively over time. These increases have been accompanied by increases in the percentage of cells that are resistant to DNA-damaging agents, indicating a reversal of the FA phenotype in the blood and bone marrow of these patients.
- *Improved vector design, stem cell selection methods, cell harvest and transduction procedures have the potential to substantially improve the quality of autologous gene therapy cell products; many of these improvements have been included in our programs*. As a result of these factors, we believe that there is reliable potential to confer disease correction at levels comparable to allogeneic transplant, but without the chemotherapy conditioning and additional side effects associated with a transplant. For example, stem cell selection methods utilized by our academic partners have increased both CD34+ cell yield and purity, while retaining select non-CD34+ populations that may be essential for successful engraftment of gene-corrected cells in the bone marrow.

Rocket Clinical Study

Efforts underway at our partners have incorporated the recommendations of an international FA working group that convened November 2010 with the intent of consolidating medical and scientific findings and optimizing future gene therapy clinical study design, with programs designed to overcome FA-specific gene therapy challenges. Our partners have demonstrated the ability to successfully mobilize, and harvest target numbers of hematopoietic stem and progenitor cells ("HSPCs") generally acknowledged to be required for successful therapy. This has been accomplished through the selection of younger patients, and mobilization with both granulocyte-colony stimulating factor ("G-CSF") and plerixa for drug products, which are both FDA-approved drugs that increase the number of bone marrow-derived stem cells circulating in the blood. Improvements to cell processing, such as reduced transduction time requirements, optimized transduction conditions, and modified HSPC selection processes, have also led to substantive improvements in cell recovery and *in vivo* VCN.

Improvements in the clinical and cell-processing components of our FA trials are expected to yield more robust and readily-identifiable disease-reversal. These improvements include selection of younger patients and identification of blood count profiles that are indicative of adequate stem cell populations capable of mobilization and engraftment in numbers sufficient for reversal of the disorder.

In contrast to the high doses of cytotoxic conditioning required for allogeneic transplant in most bone marrow disorders; our expectation is that the selective growth advantage of gene-corrected HSPCs in FA will enable treatment without conditioning agents to facilitate engraftment.

The engraftment of gene-corrected cells is likely to reduce the incidence of bone marrow failure. In addition, gene-corrected cells are likely to diminish the replicative stress in FA bone marrow, which has been increasingly implicated as a likely driver of the development or bone marrow failure or leukemia.

Each of our LVV-based programs utilize third-generation, self-inactivating lentiviral vectors 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 acute myeloid leukemia ("AML"), a type of blood cancer, as well as bone marrow failure and 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 LVV-based program targeting FA, RP-L102. RP-L102 is our lead lentiviral vector-based program that we in-licensed from Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas ("CIEMAT"), which is a leading research institute in Madrid, Spain. RP-L102 is currently being studied in our Phase 2 registrational enabling clinical trials treating FA patients at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford"), the University of Minnesota, Great Ormond Street Hospital ("GOSH") in London and Hospital Infantil de Nino Jesus ("HNJ") in Spain. The trial is expected to enroll a total of ten patients from the U.S. and EU with the first patient in this Phase 2 trial treated in December 2019. Patients will 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 2020, we presented updated interim data from our FA at the 62nd American Society of Hematology ("ASH") Annual Meeting. The FA data presented at the ASH Annual Meeting were from seven of the nine patients treated (out of twelve patients enrolled) as of October 2020 in both the U.S. Phase 1 and global Phase 2 studies of RP-L102 for FA. Patients in these studies received a single intravenous infusion of "Process B" RP-L102 which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector. Preliminary data from these studies support "Process B" as a consistent and reproducible improvement over "Process A" which was used in earlier academic FA studies.

Seven patients had follow-up data of at least two-months and three of the seven patients had been followed for twelve-months or longer. As patients are treated with gene therapy product without the use of a conditioning regimen, the data indicated that RP-L102 was generally well-tolerated with no significant safety issues reported with infusion or post-treatment. One drug related serious adverse event of Grade 2 transient infusion-related reaction was observed. In five out of the seven patients for whom there was follow-up data, evidence of preliminary engraftment was observed, with bone marrow ("BM") vector copy numbers ("VCNs") from 0.16 to 0.22 (long-term follow-up only) and peripheral VCNs ranging from 0.01 (2-month follow-up) to 0.11 (long-term follow-up). Further, two of the three patients with greater than 12-months follow-up showed evidence of increasing engraftment, mitomycin-C ("MMC") resistance and stable blood counts, which suggests a halt in the progression of bone marrow failure. The third patient with greater than 12-month follow-up contracted *Influenza B* nine months post-treatment resulting in progressive BM failure, for which, such patient received a successful bone marrow transplant at 18 months post-treatment.

In May 2021, we presented positive clinical data at the 24th Annual Meeting of the American Society of Gene and Cell Therapy ("ASGCT"). The preliminary data from the Phase 1/2 trials presented in a poster at ASGCT were from nine pediatric patients and showed increasing evidence of engraftment in at least six of the nine patients, including two patients with at least 15-months of follow-up and four patients with at least 6-months of follow-up. RP-L102 demonstrated a highly favorable tolerability profile with all subjects being treated without conditioning and with no sign of dysplasia. One patient experienced a Grade 2 transient infusion-related reaction.

In December 2021, we presented encouraging clinical data at the 63rd Annual Meeting of the American Society of Hematology (ASH). The preliminary results from the Phase 1/2 trials were presented in a poster at ASH were from eleven pediatric patients and showed increasing evidence of engraftment in at least six of eight patients for whom there are at least 12 months of follow-up, including bone marrow progenitor cell resistance to mitomycin-C (MMC) ranging from 16-63% in six patients (bone marrow cells in FA patients are highly sensitive to DNA-damaging agents including MMC; this susceptibility to DNA damage is believed to mediate the FA-associated bone marrow failure and predisposition to malignancy. In addition to the development of MMC-resistance in BM hematopoietic cells, sustained peripheral VCN levels were seen in six of seven patients with at least 12-months of follow-up. One patient experienced an Influenza B infection approximately 9 months following treatment with concomitant progressive hematologic failure requiring allogeneic hematopoietic stem cell transplant, which was administered successfully; the remaining patients have not required transfusions. RP-L102 demonstrated a highly favorable tolerability profile with all subjects being treated without cytotoxic conditioning and no signs of dysplasia. The only RP-L102 related serious adverse event to-date has been a Grade 2 transient infusion-related reaction in one patient.

Leukocyte Adhesion Deficiency-I (LAD-I):

Overview of 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.

Current Therapy

Allogeneic HSCT is the only known curative therapy, with survival rates of approximately 75% in recent studies. Allogeneic HSCT in LAD-I has been associated with frequent severe and chronic GVHD, and high rates of subsequent non-bacterial infections (most notably cytomegalovirus (“CMV”) and other viral and systemic fungal infections).

Because LAD-I is the result of mutations in a single gene (ITGB2), we are developing RP-L201, an LVV based program, to enable a potentially curative therapy utilizing patients’ own HSPCs, without the dependency on the rapid identification of an appropriate donor required in allogeneic HSCT therapy. It is anticipated that autologous therapy with RP-L201 will also enable definitive correction of this life-threatening disorder with reduced short- and long-term toxicity relative to allogeneic HSCT.

Rationale for Gene Therapy in LAD-I

We believe there are two key reasons why gene therapy could have a transformative role in the treatment of LAD-I: (1) the existence of evidence that even modest correction of the expression of the genetic mutation will increase patient survival in the severe form of the disease, and (2) consistent and robust improvements in transduction and cell processing. Of note, proprietary transduction protocols currently yield product VCNs ≥ 1 and transduction efficiencies of > 50%. In addition, with the addition of either of two transduction enhancing agents, at least a doubling of product VCN has been demonstrated in preliminary experiments.

We believe that combined with a relatively straightforward cell harvest procedure in LAD-I and the likely modest CD18 expression required for clinical impact, RP-L201 can yield a gene therapy product that confers disease resolution comparable to allogeneic HSCT, and without the severe HSCT-associated acute and chronic toxicities.

Rocket Clinical Study

We currently have one *ex*-vivo program targeting LAD-I, RP-L201. RP-L201 is a clinical program that we in-licensed from CIEMAT. We have partnered with UCLA to lead U.S. clinical development efforts for the LAD-I program. 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 serving as the lead clinical sites in Spain and London, respectively. This study has received a $6.5 million CLIN2 grant award from the California Institute for Regenerative Medicine (“CIRM”) to support the clinical development of gene therapy for LAD-I.

The ongoing open-label, single-arm, Phase 1/2 registration-enabling clinical trial of RP-L201 has treated four severe LAD-I patients to assess the safety and tolerability of RP-L201 to date. The first patient was treated at UCLA with RP-L201 in the third quarter 2019. Enrollment is now complete in both the Phase 1 and 2 portions of the study; 9 patients have received RP-L102 at 3 investigative centers in the US and Europe.

In December 2021, we presented positive clinical data at the 63rd Annual Meeting of ASH. The ASH oral presentation included preliminary data from eight of nine severe LAD-I patients, as defined by CD18 expression of less than 2%, who received RP-L201 treatment as of the November 8, 2021, data cut-off date. Eight patients had follow-up data of at least three months, and four of the eight patients had been followed for 12 months or longer. All infusions of RP-L201 were well tolerated and no drug product-related serious adverse events were reported. Evidence of preliminary efficacy was observed in all eight evaluable patients. All eight patients demonstrated neutrophil CD18 expression that exceeded the 4-10% threshold associated with survival into adulthood and consistent with reversal of the severe LAD-I phenotype including six patients with at least 6 months of follow-up. Peripheral blood VCN levels have been stable and in the 0.54 – 2.94 copies per genome range. No patients had LAD-I related infections requiring hospitalization after hematopoietic reconstitution post-RP-L201. Additional updates presented in January 2022 included a ninth patient achieving CD18 expression of 61% at 3 months, with the preliminary observation that all nine of nine patients have demonstrated 26% to 87% CD18 expression at timepoints ranging from 3 to 24 months following RP-L102, with stable CD18 expression levels for each patient subsequent to month 3.

Pyruvate Kinase Deficiency (PKD):

Overview of 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 despite the lack of FDA-approved molecularly targeted therapies. Market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 patients per year.

Current Therapy

Therapy for PKD is largely supportive, comprised of RBC transfusions and splenectomy for patients who require frequent transfusions. Chronic RBC transfusions alleviate anemia symptoms, but are associated with increased morbidity, predominantly from iron overload which may result in cirrhosis, which is a loss of liver cells and irreversible scarring of the liver, and cardiomyopathy, a chronic disease of the heart muscle that leads to a larger and bulky but inefficient heart, if not diligently managed. Iron chelation is often considered essential to offset the iron overload associated with chronic hemolysis and RBC transfusions. Iron chelation entails continuous oral or injected therapy, often for the duration of a patient's lifetime and has been associated with diminished quality of life.

Splenectomy may confer a benefit in PKD, frequently yielding increased hemoglobin ("Hb") levels of 1-2g/dL and a reduction in transfusion requirements. However, some patients do not benefit from this procedure, and it is estimated that a substantial proportion of PKD patients remain severely anemic or transfusion-dependent despite splenectomy. Splenectomy does not eliminate hemolysis, iron overload or the need for iron chelation. It also confers an increased susceptibility to serious bacterial infections, and potentially increases the risk of other PKD-associated or other complications such as venous thromboembolism and aplastic or hemolytic crises.

Allogeneic HSCT has been performed successfully for a small number of PKD patients, with reported correction of the clinical and laboratory features of the disorder. Although reports of HSCT in PKD suggest that correction of the genetic defect in hematopoietic stem cells may be curative of the disorder, HSCT requires identification of an appropriate HLA-matched donor, is associated with considerable short- and long-term complications including transplant-related mortality and is not considered a standard-of-care in PKD.

Rationale for Gene Therapy in PKD

Patients with heterozygous PKLR mutations have 50% of normal enzyme activity and are phenotypically normal. This suggests that it is not necessary for a therapy to achieve normal enzyme levels to have a clinically meaningful effect. In PKD-affected mice transplanted with normal marrow, the presence of 10% normal marrow was sufficient to restore normal red blood cells. We have conducted experiments in which bone marrow cells from healthy mice are transplanted into PKD affected mice and these results suggest that significant improvement in PKD may be achieved with 20% correction of bone marrow, and complete clinical resolution may likely be achieved when the percentage of bone marrow gene-corrected cells is in the 20 to 40% range. An additional study showed that a PKD-affected dog treated with an *ex vivo* gene therapy was rendered transfusion independent with a normalization of lactate dehydrogenase, despite only partial gene correction.

Available therapies for PKD, such as splenectomy, are generally associated with an approximate 1.5g/dL increase in hemoglobin levels, and do not modify the underlying iron overload associated with the disorder. As indicated in the pre-clinical studies noted above, and more importantly, in the initial clinical results provided subsequently, gene therapy involving genetic correction of long-term stem and progenitor cells, offers a potential for more comprehensive improvements in hemoglobin, with concomitant reduction in hemolysis and iron overload. As demonstrated in natural history studies published during recent years, the majority of patients receiving splenectomy do not achieve normal hemoglobin levels and hence there continues to be substantial need for therapies with potential to correct the underlying causes of the disorder and associated with hemoglobin corrections reaching or approaching normal range.

Rocket Clinical Study

We currently have one *ex*-vivo LVV-based program targeting PKD, RP-L301. RP-L301 is a clinical stage program that we in-licensed from CIEMAT. The IND for RP-L301 to initiate the global Phase 1 study cleared in October 2019. This program has been granted US and EMA orphan drug disease designation.

This global Phase 1 open-label, single-arm, clinical trial is expected to enroll six adult and pediatric PKD patients in the U.S. and Europe. The trial will be comprised of three cohorts to assess RP-L301 in young pediatric (age 8-11), older pediatric (age 12-17) and adult populations. The trial is designed to assess the safety, tolerability, and preliminary activity of RP-L301, and initial safety evaluation will occur in the adult cohort before evaluation in pediatric patients. Stanford will serve as the lead site in the U.S. for adult and pediatric patients, HNJ will serve as the lead site in Europe for pediatrics, and Hospital Universitario Fundación Jiménez Díaz will serve as the lead site in Europe for adult patients. In July 2020, we treated the first patient in our clinical trial of RP-L301.

In December 2021, we presented positive clinical data at the 63rd Annual Meeting of ASH. The ASH poster presentation included preliminary data from two adult patients with severe anemia and substantial transfusion requirements who were treated as of the November 3, 2021 cut-off date. Each of these patients had experience extensive PKD-related disease complications including hepatic iron overload. Both patients have had marked improvement in hemoglobin levels, from baselines of 7.4 and 7.0 g/dL to 12-month values of 13.3 and 14.8 g/dL respectively; this represents an improvement from severe (Hb <8g/dL) to normal levels. Both patients have been transfusion independent subsequent to post-treatment hematopoietic reconstitution. Anemia resolution has been accompanied by marked improvement in additional markers of hemolysis, including bilirubin, erythropoietin, and reticulocyte counts. RP-L301 has been well tolerated in these adult patients, with no drug product related serious adverse events or infusion-related complications observed through 12-months post-treatment. Both patients have reported improved quality of life (QOL) following treatment with increases on FACT-An and additional designated QOL evaluations sustained through 12 months following therapy.

Infantile Malignant Osteopetrosis (IMO):

Overview of Infantile Malignant Osteopetrosis

IMO is a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. During normal growth and development small areas of bone are constantly being broken down by special cells called osteoclasts, then made again by cells called osteoblasts. In IMO, the cells that break down bone (osteoclasts) do not work properly, which leads to the bones becoming thicker and not as healthy. Untreated IMO patients may suffer from a compression of the bone-marrow space, which results in bone marrow failure, anemia, and increased infection risk due to the lack of production of white blood cells. Untreated IMO patients may also suffer from a compression of cranial nerves, which transmit signals between vital organs and the brain, resulting in blindness, hearing loss and other neurologic deficits.

IMO represents the autosomal recessive, severe variants of a group of disorders characterized by increased bone density and bone mass secondary to impaired bone resorption. IMO typically presents in the first year of life and is associated with severe manifestations leading to death within the first decade of life in the absence of allogeneic HSCT, although HSCT results have been limited to-date and notable for frequent graft failure, GVHD and other severe complications.

Approximately 50% of IMO results from mutations in the TCIRG1 gene, resulting in cellular defects that prevent osteoclast bone resorption. As a result of this defect, bone growth is markedly abnormal. It is estimated that IMO occurs in 1 out of 250,000-300,000 within the general global population, although incidence is higher in specific geographic regions including Costa Rica, parts of the Middle East, the Chuvash Republic of Russia, and the Vasterbotten Province of Northern Sweden.

Effective December 2021, the Company made a decision to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program is to be returned to academic innovators. The Company has opted to focus available resources towards advancement of RP-A501, RP-L102, RP-L201 and RP-L301, based on the compelling clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

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, we expect to submit our first biologics license applications (“BLAs”) 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, 2022, 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 Fanconi Anemia program includes two in-licensed patent families. The first family includes pending patents and pending applications in the U.S., Europe, Japan, China and other countries with claims directed to polynucleotide cassettes and expression vector compositions containing Fanconi Anemia complementation group genes and methods for using such vectors to provide gene therapy in mammalian cells for treating Fanconi Anemia. This application was 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.

The second family includes pending U.S., Japanese, and European patent applications related to a portable platform for use in hematopoietic stem/progenitor cell-based gene therapy. This patent family was exclusively in-licensed from the Fred Hutchinson Cancer Research Center. 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 2036, absent any patent term adjustments or extensions.

Pyruvate Kinase Deficiency (PKD)

Our PKD patent portfolio includes 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. This application was 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, absent any patent term adjustments or extensions.

Danon Disease

Our Danon disease patent portfolio includes both proprietary intellectual property and a patent family in-licensed from the University of California, San Diego, which includes patent applications in the U.S., Europe, Japan, China and other countries with claims directed to the treatment of Danon disease. 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 a U.S. patent and pending patent application in the EU, Japan, China and other countries with claims directed to gene therapy vectors for the treatment of Danon disease; the U.S. patent issued in 2020. Any patents, if issued, arising from any national stage applications filed 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 a provisional patent application directed to methods for treatment of Danon disease. Any patents, if issued, arising from this provisional patent application, are expected to expire in 2041, absent any patent term adjustments or extensions, if the appropriate maintenance, renewal, annuity, or other governmental fees are paid.

Infantile Malignant Osteopetrosis (IMO)

Our IMO patent portfolio includes a pending PCT patent application with claims related to a method of manufacturing lentiviral vector used to transduce allogeneic HSCT. We expect any patents arising from any national stage applications filed from this PCT application, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2040, absent any patent term adjustments or extensions.

Leukocyte Adhesion Deficiency (LAD-I)

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.

Our objective is to continue to expand its 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 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 is 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 lentiviral vectors 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 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) 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 lentiviral vectors containing the FA-A gene solely within the field of human therapeutic uses of VSV-G packaged integration component lentiviral vectors 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) 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 €0.1 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"), collectively referred to as ("Licensors"), granting us worldwide, exclusive rights to certain patents, know-how and other intellectual property relating to lentiviral vectors 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) to Licensors for non-commercial use any improvements to the licensed intellectual property that we create.

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 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 Danon Disease 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 Danon disease. 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.

The upfront license fee of $0.05 million was expensed to R&D costs in the 2020 consolidated statements of operations. We 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 Danon disease. 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 Danon disease 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 statements 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 Danon patient in 2019 which was expensed to R&D costs in the 2019 consolidated statements of operations. There were no additional milestones achieved or related payments made during the years ended December 31, 2021 and 2020.

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 it 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 LVV vectors and AAV vectors and the target cell transduction process, which results in drug product. We will commence 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 LVV 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. Rocket’s drugs 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 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 United States 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 United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of institutional biosafety committees("IBC's"), 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 BLA's. 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 U.S. Patent and Trademark Office, 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. Bio similarity, 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. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference 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 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 United States. 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 of life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug or biological product for such disease or condition will be received from sales in the United States 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. Prescription Drug Marketing Act, a 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 racing requirements and for notifying 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 or withdrawal of the product from the market. 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 also may require the implementation of other risk management measures. Also, 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 of 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, which might not include all of the 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 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. 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 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, the ACA codified case law that a claim including items or services resulting from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the 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, 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, 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. 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.

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 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.

Healthcare Legislative Reform

In both the United States 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; and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment, there have been judicial, Congressional, and executive challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how other healthcare reform measures of the Biden administration or other efforts, if any, to challenge, repeal or replace the ACA will impact our business.

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

- 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. Pursuant to the Coronavirus Aid, Relief, and Economic Security Act, also known as the CARES Act, as well as subsequent legislation, these reductions have been suspended from May 1, 2020 through March 31, 2022 due to the COVID-19 pandemic. Then, a 1% payment reduction will occur beginning April 1, 2022 through June 30, 2022, and the 2% payment reduction will resume on July 1, 2022.
- In January 2013, the American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.
- 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.
- On December 20, 2019, former President Trump signed into law the Further Consolidated Appropriations Act (H.R. 1865), which repealed the Cadillac tax, the health insurance provider tax, and the medical device excise tax. It is impossible to determine whether similar taxes could be instated in the future.

There has been increasing legislative and enforcement interest in the United States 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. Further, on November 20, 2020 CMS issued an Interim Final Rule implementing the Most Favored Nation, or MFN, Model under which Medicare Part B reimbursement rates would have been calculated for certain drugs and biologicals based on the lowest price drug manufacturers receive in Organization for Economic Cooperation and Development countries with a similar gross domestic product per capita. However, on August 6, 2021 CMS announced a proposed rule to rescind the Most Favored Nations rule. 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, 2026. 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.

In addition, there have been several changes to the 340B drug pricing program, which imposes ceilings on prices that drug manufacturers can charge for medications sold to certain health care facilities. On December 27, 2018, the District Court for the District of Columbia invalidated a reimbursement formula change under the 340B drug pricing program, and CMS subsequently altered the FYs 2019 and 2018 reimbursement formula on specified covered outpatient drugs ("SCODs"). The court ruled this change was not an "adjustment" which was within the Secretary's discretion to make but was instead a fundamental change in the reimbursement calculation. However, most recently, on July 31, 2020, the U.S. Court of Appeals for the District of Columbia Circuit overturned the district court's decision and found that the changes were within the Secretary's authority. On September 14, 2020, the plaintiffs-appellees filed a Petition for Rehearing En Banc (i.e., before the full court), but was denied on October 16, 2020. Plaintiffs-appellees filed a petition for a writ of certiorari at the Supreme Court on February 10, 2021. On Friday July 2, 2021, the Supreme Court granted the petition. It is unclear how these developments could affect covered hospitals who might purchase our future products and affect the rates we may charge such facilities for our approved products in the future, if any. Although a number of these, and other proposed measures will require authorization through additional legislation to become effective, Congress has indicated that it may continue to seek new legislative and/or administrative measures to control drug costs.

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 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 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 Information System. 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. If the sponsor chooses to submit under the Clinical Trials Directive, the clinical trial continues to be governed by the Directive 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.

Marketing Authorization

In the European Union, 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.

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.

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 a period of two years from January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or MHRA, the UK medicines regulator, 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. A separate application will, however, still be required.

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. However, the EU and the UK have 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 implemented EU legislation on the marketing, promotion and sale of medicinal products through the Human Medicines Regulations 2012 (as amended) (under the Northern Ireland Protocol, the EU regulatory framework will continue to apply in Northern Ireland). The regulatory regime in Great Britain therefore largely aligns with current EU regulations, however it is possible that these regimes will diverge in future now that Great Britain's regulatory system is independent from the EU and the TCA does not provide for mutual recognition of UK and EU pharmaceutical legislation. For example, the UK has implemented the now repealed Clinical Trials Directive 2001/20/EC into national law through the Medicines for Human Use (Clinical Trials) Regulations 2004 (as amended). The extent to which the regulation of clinical trials in the UK will mirror the new Clinical Trials Regulation now that has come into effect is not yet known, however the Medicines and Healthcare products Regulatory Agency ("MHRA"), the UK medicines regulator, has opened a consultation on a set of proposals designed to improve and strengthen the UK clinical trials legislation. Such consultation is open until 14 March 2022.

Human Capital

As of December 31, 2021, we had 151 full-time employees, of whom 148 were located in the United States, two in Spain and one in Switzerland. We also leverage temporary workers to provide flexibility for our business needs. None of our employees are represented by a labor union or covered by a collective bargaining agreement.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing, 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 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 the creation of a work environment where our employees can feel inspired to deliver their workplace best every day. All employees are responsible for upholding the Rocket Behaviors and the Rocket Code of Conduct, which form the foundation of our policies and practices.

Corporate Information

We were incorporated in Delaware in 1999 as Inotek Pharmaceuticals Corporation. 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 (646) 440-9100. 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 Securities and Exchange Commission ("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. In these circumstances, the market price of our common stock could decline.

Risks Related to Current Novel Coronavirus (COVID-19) Pandemic

The outbreak of the novel strain of coronavirus, SARS-CoV-2, which causes COVID-19, could adversely impact our business, including our preclinical and clinical studies.

Public health crises such as pandemics or similar outbreaks could adversely impact our business. Throughout 2021 and into 2022, COVID-19 has spread extensively throughout the world and in the United States. In response to the spread of COVID-19 and its variants, most of our corporate employees converted to working remotely part-time, with a smaller number of employees whose roles require them to be on-site full-time working at our Cranbury, NJ facility.

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;
- interruption of clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures (particularly any procedures that may be deemed non-essential), which may impact the integrity of subject data and clinical study endpoints;
- delays or difficulties in the buildout of our in-house manufacturing;
- 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 comparable foreign regulatory agencies, which may impact approval timelines.

These and other factors arising from the COVID-19 pandemic could worsen in areas that are already afflicted with COVID-19, could continue to spread to additional areas, or could return to areas where the pandemic has been partially contained, each of which could further adversely impact our ability to conduct clinical trials and our business generally, and could have a material adverse impact on our operations and financial condition and results.

The extent to which the outbreak may impact our business, preclinical studies and clinical trials will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the identification of new variants of the virus, the duration of the outbreak, travel restrictions and actions to contain the outbreak or treat its impact, such as social distancing and quarantines or lock-downs in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease. In addition, a recession, depression, or other sustained adverse market event resulting from the spread of the coronavirus could materially and adversely affect our business and the value of our common stock.

Since the beginning of the COVID-19 pandemic, three vaccines for COVID-19 have been granted Emergency Use Authorization by the FDA, and two of those later received marketing approval. Additional vaccines may be authorized or approved in the future. The resultant demand for vaccines and potential for manufacturing facilities and materials to be commandeered under the Defense Production Act of 1950, or equivalent foreign legislation, may make it more difficult to obtain materials or manufacturing slots for the products needed for our clinical trials, which could lead to delays in these trials.

The ultimate impact of the current pandemic, or any other health epidemic, is highly uncertain and subject to change. We do not yet know the full extent of potential delays or impacts on our business, our clinical and preclinical programs, our clinical, preclinical, research, manufacturing, and regulatory activities, healthcare systems or the global economy. However, these effects could have a material adverse impact on our operations, and we will continue to monitor the situation closely.

Risks Related to Our Financial Condition and Capital Needs

Risks Related to Our Financial Condition

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 an early-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 $169.1 million, $139.7 million and $77.3 million for the years ended December 31, 2021, 2020 and 2019, respectively. As of December 31, 2021, we had an accumulated deficit of $491.9 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 over the next several years and for the foreseeable future as we intend to continue to conduct R&D, clinical trials, regulatory compliance activities, internal and external manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated G&A expenses, will likely result in us continuing to incur significant losses for the foreseeable future.

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 completed clinical trials of our product candidates, 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 may need to transition to a commercial stage company. We cannot guarantee that we will be successful in this transition.

Changes in tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

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 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. 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 NOLs 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. Future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the Internal Revenue Code and limit our ability to utilize our NOLs and credits. Our NOLs or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or credits if we undergo an ownership change prior to the utilization of all such NOLs 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. Furthermore, we expect to incur additional costs associated with operating as a public company, particularly as we no longer qualify as an emerging growth company or smaller reporting company and are subject to certain disclosure and compliance requirements as a large accelerated filer that we were not previously subject to. 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 capital by issuing equity securities, our stockholders will experience substantial additional dilution.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2021, our cash, cash equivalents and investments were $388.7 million. Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement, completion, and results of our preclinical studies and clinical trials;
- costs for seeking regulatory approval for our product candidates in the U.S., Europe and other jurisdictions;
- the production of LVV and AAV gene therapy product candidates to support preclinical and clinical needs;
- the results of our preclinical studies for our current product candidates and any subsequent clinical trials;

- the scope, progress, results, and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the costs, timing, and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing clinical-grade and commercial-grade product to support our clinical trials and commercial launch, if approved;
- the ability to receive additional non-dilutive funding, including grants from organizations and foundations;
- the revenue, if any, received from commercial sale of its products, should any of its product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the timing, receipt, and amount of sales of, or milestone payments related to our royalties on, current or future product candidates, if any.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

To the extent that additional capital is raised through the sale of equity or equity-linked securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

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 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 have initiated Rocket-sponsored clinical trials for FA, LAD-I, PKD and Danon disease, but have not completed any clinical trials to date. 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 at domestic or foreign clinical sites;
- a shutdown of the U.S. government, including the FDA;
- public health crises such as pandemics and epidemics; or
- natural disasters at any of our clinical sites.

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 CROs and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of LVV 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;
- design of the study protocol;
- 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;
- efforts to facilitate timely enrollment in 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 LVV 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.

We have not completed any clinical studies of our current product candidates. 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 have initiated our sponsored clinical trials for FA, LAD-I, PKD and DD but have not completed any clinical trials to date. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. 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. Positive data may not continue or occur for subjects in our clinical studies or for any future subjects in our ongoing or future clinical studies and may not be repeated or observed in ongoing or future studies involving our product candidates. Furthermore, our product candidates may also 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. From time to time, we may also disclose interim data from our clinical trials. 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 commercialization 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. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction 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 Danon Disease 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. 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. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

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:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials; and
- our reputation may suffer.

Any of these occurrences 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, only a few gene and cell therapy products have been approved in the United States and the European Union.

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. We cannot guarantee that we will not experience problems or delays in developing current or future product candidates or that such problems or delays will not cause unanticipated costs, or that any such development problems or delays can be resolved. We may also experience unanticipated problems or delays in developing our manufacturing capacity or transferring our manufacturing process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a 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 and Spark Therapeutics' Luxturna. 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 Center for Biologics Evaluation and Research ("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. For example, the agency has released a series of draft and final guidance documents relating to, among other topics, various aspects of gene therapy product development, review, and approval, including aspects relating to clinical and manufacturing issues related to gene therapy products. In January 2020, FDA released a 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 lentiviral vectors, 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 United States, 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 United States 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 United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, 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 European Union; or (ii) without incentives, it is unlikely that sales of the drug in the European Union 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 European Union 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 Fanconi Anemia, for RP-L201 for the treatment of Leukocyte Adhesion Deficiency-1, for RP-L301 for the treatment of Pyruvate Kinase Deficiency, and FDA orphan drug designation for RP-A501 for treatment of Danon Disease and RP-401 for the treatment of Infantile Malignant Osteopetrosis. 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), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, 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 United States. 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. 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 European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;

- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

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 Danon disease, RP-L102 for FA, RP-L201 for LAD-I and RP-L301 for PKD, and 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. Marketing applications filed by sponsors of products in fast-track development may qualify for priority review under the policies and procedures offered by the FDA.

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-L102 for FA and RP-L201 for LAD-I. 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 and RP-L201 for LAD-I. 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.

In the European Union, 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 European Union 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 Danon disease, 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 Danon disease, 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.

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. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. 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 GMP, and current good tissue practice, 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 United States. If we or a regulatory agency discover previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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

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

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

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.

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. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. 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. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory approval process involves similar risks to those associated with FDA and EMA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, nor have we attempted to obtain such approval. 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.

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

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or 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 is still being interpreted and implemented by the FDA. 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. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. 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 United States 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 United States 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 United States 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 European Union 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 transitional period, during which EU laws, including pharmaceutical laws, continued to apply in the UK, however this ended on December 31, 2020. The UK reached a trade agreement with the European Union on December 24, 2020, which became provisionally applicable on January 1, 2021 and has been formally applicable since May 1, 2021. Under the terms of the deal, the EU and UK have separate regulatory regimes for pharmaceutical products, although there are some provisions for mutual recognition of standards, for example with regards to GMP. For instance, the UK is no longer covered by the centralized procedure for obtaining EU-wide marketing authorizations for medicinal products, and a separate process for authorization of medicinal products will be required in the UK, resulting in an authorization covering the UK or Great Britain (England, Scotland and Wales) only. At present, Great Britain has implemented EU legislation on the marketing, promotion and sale of medicinal products through the Human Medicines Regulations 2012 (as amended) (under the Northern Ireland Protocol, the EU regulatory framework will continue to apply in Northern Ireland). The regulatory regime in Great Britain therefore currently broadly aligns with EU regulations, however it is possible that these regimes will diverge in future now that Great Britain's regulatory system is independent from the EU and the TCA does not provide for mutual recognition of UK and EU pharmaceutical legislation.

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 European Union 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.

In addition, as a result of Brexit, other European countries may seek to conduct referenda with respect to their continuing membership with the European Union. Given these possibilities and others we may not anticipate, as well as the absence of comparable precedent, it is unclear what financial, regulatory and legal implications the withdrawal of the United Kingdom from the European Union would have and how such withdrawal would affect us, and the full extent to which our business could be adversely affected.

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.

If we fail to comply with applicable U.S. and foreign privacy and data protection laws and regulations, we may be subject to liabilities that adversely affect our business, operations and financial performance.

We may also be subject to or affected by foreign laws and regulation, including regulatory guidance, governing the collection, use, disclosure, security, transfer and storage of personal data, such as information that we collect about patients and healthcare providers in connection with clinical trials and our other operations in the U.S. and abroad. For example, the E.U.'s General Data Protection Regulation ("GDPR"), which introduces strict requirements for processing all personal data and imposes even more stringent requirements for certain categories of sensitive information, including health related information. The GDPR increases the compliance burden on us, including by mandating potentially burdensome documentation requirements and granting certain rights to individuals to control how we collect, use, disclose, retain and leverage information about them. The processing of sensitive personal data, such as health information, imposes heightened compliance burdens under the GDPR and is a topic of active interest among foreign regulators. In addition, the GDPR provides for breach reporting requirements, more robust regulatory enforcement and fines of up to 20 million euros or up to 4% of annual global revenue. While the GDPR affords some flexibility in determining how to comply with the various requirements, significant effort and expense has been, and will continue to be, invested to ensure continuing compliance. Moreover, the requirements under the GDPR may change periodically or may be modified by EU national law and could affect our business operations if compliance becomes substantially more costly than under current requirements.

In addition, further to the U.K.'s exit from the EU on January 31, 2020, the GDPR ceased to apply in the U.K. at the end of the transition period on December 31, 2020. However, as of January 1, 2021, the U.K.'s European Union (Withdrawal) Act 2018 incorporated the U.K. GDPR into U.K. law. The U.K. GDPR and the U.K. Data Protection Act 2018 set out the U.K.'s data protection regime, which is independent from but aligned to the EU's data protection regime. Non-compliance with the U.K. GDPR may result in monetary penalties of up to £17.5 million or 4% of worldwide revenue, whichever is higher. Although the UK is regarded as a third country under the EU's GDPR, the European Commission ("EC") has now issued a decision recognizing the UK as providing adequate protection under the EU GDPR and, therefore, transfers of personal data originating in the EU to the UK remain unrestricted. Like the EU GDPR, the UK GDPR restricts personal data transfers outside the UK to countries not regarded by the UK as providing adequate protection. The UK government has confirmed that personal data transfers from the UK to the EEA remain free flowing.

To enable the transfer of personal data outside of the EEA or the UK, adequate safeguards must be implemented in compliance with European and UK data protection laws. On June 4, 2021, the EC issued new forms of standard contractual clauses for data transfers from controllers or processors in the EU/EEA (or otherwise subject to the GDPR) to controllers or processors established outside the EU/EEA (and not subject to the GDPR). The new standard contractual clauses replace the standard contractual clauses that were adopted previously under the EU Data Protection Directive. The UK is not subject to the EC's new standard contractual clauses but has published a draft version of a UK-specific transfer mechanism, which, once finalized, will enable transfers from the UK. We will be required to implement these new safeguards when conducting restricted data transfers under the EU and UK GDPR and doing so will require significant effort and cost.

In the United States, there are numerous privacy laws that may be applicable to our activities, and a range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. New laws also are being considered or have been implemented at both the state and federal levels. For example, the California Consumer Privacy Act of 2018, or the CCPA, which became effective on January 1, 2020, requires companies that process information of California residents to make new disclosures to consumers about their data collection, use and sharing practices, provides Californian consumers (as defined under the CCPA) with new individual data privacy rights, imposes new operational requirements for covered businesses, provides a private right of action for data breaches and creates a statutory damages framework. 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 how such laws are interpreted. Additionally, effective starting on January 1, 2023, the California Privacy Rights Act, or the CPRA, will significantly modify the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that will be vested with authority to implement and enforce the CCPA and the CPRA. Certain states, including Colorado and Virginia, have enacted laws similar to the CCPA and many other states are considering similar legislation, and a broad range of legislative measures also have been introduced at the federal level.

Additionally, some observers have noted that the CCPA and CPRA could mark the beginning of a trend toward more stringent privacy legislation in the U.S., which could increase our potential liability and adversely affect our business. Already, in the United States, we have witnessed significant developments at the state level. For example, on March 2, 2021, Virginia enacted the Consumer Data Protection Act (the "CDPA"), and, on July 8, 2021, Colorado's governor signed the Colorado Privacy Act ("CPA"), into law. The CDPA and the CPA will both become effective January 1, 2023. While the CDPA and CPA incorporate many similar concepts of the CCPA and CPRA, there are also several key differences in the scope, application, and enforcement of the law that will change the operational practices of regulated businesses. The new laws will, among other things, impact how regulated businesses collect and process personal sensitive data, conduct data protection assessments, transfer personal data to affiliates, and respond to consumer rights requests.

A number of other states have proposed new privacy laws, some of which are similar to the above discussed recently passed laws. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. The existence of comprehensive privacy laws in different states in the country would make our compliance obligations more complex and costly and may increase the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance.

Further, regulations promulgated pursuant to HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their implementing regulations, or collectively HIPAA, imposes privacy, security and breach notification obligations on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, as well as their business associates that perform certain services that involve creating, receiving, maintaining or transmitting individually identifiable health information for or on behalf of such covered entities, and their covered subcontractors. HIPAA establishes privacy and security standards that limit the use and disclosure of individually identifiable health information and protected health information, or PHI, and requires the implementation of administrative, physical and technological safeguards to protect the privacy of PHI and ensure the confidentiality, integrity and availability of electronic PHI. Most healthcare providers, including research institutions from which we obtain patient health information, are privacy and security regulations promulgated under HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act. We do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information.

The global legislative and regulatory landscape for privacy and data protection continues to evolve, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. This evolution may create uncertainty in our business, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future.

It is possible that privacy laws may be interpreted and applied in a manner that is inconsistent with our practices. Any failure or perceived failure by us to comply with federal, state, or foreign laws or self-regulatory standards could result in negative publicity, diversion of management time and effort and proceedings against us by governmental entities or others. In many jurisdictions, enforcement actions and consequences for noncompliance are rising. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems. Further, any failure by our third-party collaborators, service providers, contractors or consultants to comply with applicable law, regulations or contractual obligations related to data privacy or security could result in proceedings against us by governmental entities or others.

We may also publish privacy policies and other documentation regarding our collection, processing, use and disclosure of personal information and/or other confidential information. Although we endeavor to comply with our published policies and other documentation, we may at times fail to do so or may be perceived to have failed to do so. Despite our efforts, we may not be successful in achieving compliance if our employees or vendors fail to comply with our published policies and documentation. Such failures can subject us to potential international, local, state and federal action if they are found to be deceptive, unfair, or misrepresentative of our actual practices. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy notices, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. We also face a threat of consumer class actions related to these laws and the overall protection of personal information. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business, financial condition, results of operations or prospects

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 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 our product candidates. 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. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority finds deficiencies with or does not approve these facilities for the manufacture of our product candidates or if it finds deficiencies or withdraws any such approval in the future, we may need to find alternative manufacturing facilities, 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 are preparing our manufacturing facility for cGMP manufacturing. We have no experience in manufacturing, and there can be no assurance that we will be able to manufacture products.

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 are now preparing the facility to be ready for cGMP manufacturing in 2022.

Although some of our employees have experience in the manufacturing of biopharmaceutical products from prior employment at other companies, we as a company have no prior experience in manufacturing. In addition, government approvals will be required for us to operate a manufacturing facility and can be time-consuming to obtain, and there can be no assurance that such approval will be obtained. As a manufacturer of pharmaceutical products, we also 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.

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.

In the United States and markets in other countries, patients generally rely on third-party payors to reimburse all, or part of the costs associated with their treatment. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford expensive 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. We cannot guarantee that reimbursement will be available for any of our product candidates. 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. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If reimbursement is not available, or is available only at limited levels, we may not be able to successfully commercialize our product candidates, if approved. 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*".

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the U.S., the principal decisions about coverage and reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or 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 are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In recent years, numerous proposals to change the health care system in the U.S. have been made. These reform proposals include measures that would limit or prohibit payments for certain medical treatments or subject the pricing of drugs to government control. In addition, in many foreign countries, particularly the countries of the EU, the pricing of prescription drugs is subject to government control. If our products are or become subject to government regulation that limits or prohibits payment for our products, or that subjects the price of our products to governmental control, we may not be able to generate revenue, attain profitability or commercialize our products.

In addition, 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 United States. 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. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price, or ASP, and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs. If we are unable to obtain adequate levels of reimbursement for our product candidates, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important to successful commercialization of our product candidates. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

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 European Union 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 European Union do not follow price structures of the U.S. and generally prices tend to be significantly lower.

Even if approved, we may not successfully commercialize our product candidates.

Our gene therapy product candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe, commercially viable products would severely limit our ability to become profitable or to achieve significant revenues. Even if one or more of our product candidates is approved, we may be unable to successfully commercialize our product candidates for several reasons, including:

- some or all of our product candidates may be found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances; our product candidates, if safe and effective, may nonetheless not be able to be developed into commercially viable products;
- it may be difficult to manufacture or market our product candidates on a scale that is necessary to ultimately deliver our products to end-users;
- proprietary rights of third parties may preclude us from marketing our product candidates;
- the nature of our indications as rare diseases means that the potential market size may be limited; and
- third parties may market superior or equivalent drugs which could adversely affect the commercial viability and success of our product candidates.

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, manufacturing capabilities and 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 by physicians, patients, third-party payors and others in the medical community.

Ethical, social, legal and other concerns about gene therapy could result in additional regulations restricting or prohibiting our products. 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. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If any such products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of 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;
- 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 awareness of, and willingness to seek, gene therapy;
- the willingness of physicians to prescribe new therapies;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- 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.

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 research and development 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, 2021. 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 time frames 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 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.

If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data, they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our business, financial condition and results of operations and the commercial prospects for our product candidates could be materially and adversely affected, our costs could increase, and our ability to generate revenue could be delayed.

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 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.

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

Our reliance on these third parties for R&D activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies required to support future product submissions and approval of our product candidates.

Generally, these third parties may terminate their engagements with us at will upon notice. If we need to enter into alternative arrangements, it could delay our product development activities.

We expect to rely on third-party manufacturers to manufacture supplies of certain of our product candidates, including all of the LVV product candidates. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured all the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for certain aspects of manufacturing activities;
- the risk that these activities are not conducted in accordance with our study plans and protocols;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical study delays or failure to obtain regulatory approval or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including an injunction, recall, seizure or total or partial suspension of production.

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.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on third parties to manufacture our product candidates and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our gene therapy platform, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor’s discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

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, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA 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 ongoing 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 and due to the COVID-19 pandemic and travel restrictions the FDA is unable to complete such required inspections during the review period. Since March 2020 when foreign and domestic inspections of facilities were largely placed on hold, the FDA has been working to resume routine surveillance, bioresearch monitoring and pre-approval inspections on a prioritized basis. Since April 2021, the FDA has conducted limited inspections and employed remote interactive evaluations, using risk management methods, to meet user fee commitments and goal dates. Ongoing travel restrictions and other uncertainties continue to impact oversight operations both domestic and abroad and it is unclear when standard operational levels will resume. The FDA is continuing to complete mission-critical work, prioritize other higher-tiered inspectional needs (e.g., for-cause inspections), and carry out surveillance inspections using risk-based approaches for evaluating public health. 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 has stated that it generally intends to issue, depending on the circumstances, 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 ongoing COVID-19 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

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. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

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

If we are unable to obtain and maintain patent protection for 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. Our licensors have sought, and we may intend to seek, 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.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates may expire prior to commercial launch of our products; this patent expiration risk could be partially addressed 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. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work. It is also possible that we will fail to identify patentable aspects of our R&D output before it is too late to obtain patent protection.

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 fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we could lose certain rights provided by the licenses, including our ability to market products covered by the license.

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

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. For example, Institut Pasteur controls a patent family related to vector elements for lentiviral-based gene therapy. These patents relate to an element that improves nuclear localization. While these patents began expiring in 2019, and will entirely expired by 2023, if our products were to launch before the fourth quarter of 2023, we may need to secure a license. In addition, 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.

If we 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, and what activities satisfy those diligence obligations;
- 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 United States 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.

If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own, or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to patents and patent applications owned or in-licensed by us have been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Furthermore, even if they are unchallenged, patents and patent applications that we own or in-license may not adequately protect our intellectual property, provide exclusivity for our product candidates or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products. In addition to our existing patent application filings, we expect to continue to file additional patent applications covering our product candidates. Further, we intend to pursue additional activities to protect the patents, trade secrets and other intellectual property covering our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we or the relevant licensor encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or the relevant licensor were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be initiated by a third-party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available however 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 for a product, we may be open to competition from generic medications.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on 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 secret. In addition, others may independently discover our trade secrets and proprietary information.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property, both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, it may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

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.

Presently we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene therapy product candidates. 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 are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

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 time frame or under terms that are acceptable to it. 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 U.S. PTO and various governmental patent agencies outside of the United States 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 U.S. PTO 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 United States, 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 U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, 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. 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 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.

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

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

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 United States, 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 research and development 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.

Risks Related to Claims Arising from our Intellectual Property

We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights and we may be unable to protect our rights to, or use, our technology.

If we choose to engage in legal action to prevent a third-party from using the inventions claimed in our patents or patents which we license, that third-party has the right to ask the court to rule that these patents are invalid and/or should not be enforced against that third-party. These lawsuits are expensive and would consume time and other resources even if we were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we do not have the right to stop the other party from using the inventions. There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to these patents.

Furthermore, a third-party may claim that we are using inventions covered by the third-party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and technical personnel. There is a risk that a court would decide that we are infringing the third-party's patents and would order us to stop the activities covered by the patents. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. The biotechnology industry has produced a proliferation of patents, and it is not always clear to industry participants which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our products or methods of use either do not infringe the patent claims of the relevant patent and/or that the patent claims are invalid. Proving invalidity, in particular, is difficult since it requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Our competitors have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our in-licensed patent applications and could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the U.S. Patent and Trademark Office, to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful, resulting in a loss of our United States patent position with respect to such inventions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

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.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, ex parte reexaminations, post-grant review, and *inter partes* review proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products, or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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 Chief Medical Officer and Head of Clinical Development, Jonathan Schwartz, MD; our President and Chief Operating Officer, Kinnari Patel, PharmD, MBA; and our Chief Financial Officer, Carlos Garcia-Parada. 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.

Given our commercial relationships outside of the United States, in particular in the European Union, 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 United States, 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 United States;
- 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 European Union General Data Privacy Regulation (“GDPR”); and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

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

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

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. 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 acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with any anticipated business or product acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of R&D efforts;
- retention of key employees from any acquired company;
- changes in relationships with strategic partners as a result of any product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from any acquired company into our organization or managing a strategic alliance or joint venture;
- the need to implement or improve controls, procedures, and policies at any acquired business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of any acquired company before the acquisition, 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 in connection with any acquired company, including claims from terminated 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 acquisitions or 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 acquisitions will 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.

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 have entered or 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 United States 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;
- 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.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

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 25.2% 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.

Other Risks Related to Our Business

As of December 31, 2020, we were no longer an "emerging growth company," as defined in the JOBS Act. or a "smaller reporting company" as defined in the Exchange Act and were a "large accelerated filer" which subjects us to increased disclosure and compliance requirements.

As a large accelerated filer, we are subject to certain disclosure and compliance requirements that apply to other public companies but did not historically apply to us due to our prior status as an emerging growth company. These requirements include, but are not limited to:

- the requirement that our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act of 2002;
- compliance with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- the requirement that we provide full and more detailed disclosures regarding executive compensation; and
- the requirement that we hold a non-binding advisory vote on executive compensation and obtain stockholder approval of any golden parachute payments not previously approved.

We expect that continued compliance with the requirements of being a large accelerated filer may increase our legal and financial compliance costs and cause management and other personnel to divert attention from operational and other business matters to devote substantial time to public company reporting requirements. In addition, if we are not able to comply with changing requirements in a timely manner, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC, or other regulatory authorities, which would require additional financial and management resources.

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. Beginning in 2020, we will be required to furnish a report by management on the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act ("Section 404"). Pursuant to Section 404 our independent registered public accounting firm will be 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 may increase our expenses and require significant management time. Investors may find our common stock less attractive because of the additional compliance costs. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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. Internal control deficiencies could also result in a restatement of our financial results in the future. We could become subject to stockholder or other third-party litigation, as well as investigations by the SEC, the New York Stock Exchange, 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.

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

Social media is increasingly being used to communicate about our clinical development programs and the diseases our product candidates are being developed to treat. We intend to utilize appropriate social media in connection with our commercialization efforts following approval of our product candidates. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations, or we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our investigational products. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website, or a risk that a post on a social networking website by any of our employees may be construed as inappropriate promotion. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

Item 1B. Unresolved SEC Comments

None.

Item 2. Properties

Corporate Headquarters, R&D and GMP Manufacturing 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 Current Good Manufacturing Practice (cGMP) manufacturing to support our pipeline. A smaller area within this facility was originally leased in August 2018, and the lease was amended in June 2019 to include the full building (such lease, as amended, the NJ Lease Agreement) The NJ Lease Agreement has an initial term which ends 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.

Facility in Lexington, Massachusetts

We currently lease approximately 15,000 square feet of office space in Lexington, Massachusetts under a lease that expires in February 2023. This space is the former headquarters of Inotek and is subleased through the remainder of the lease term.

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

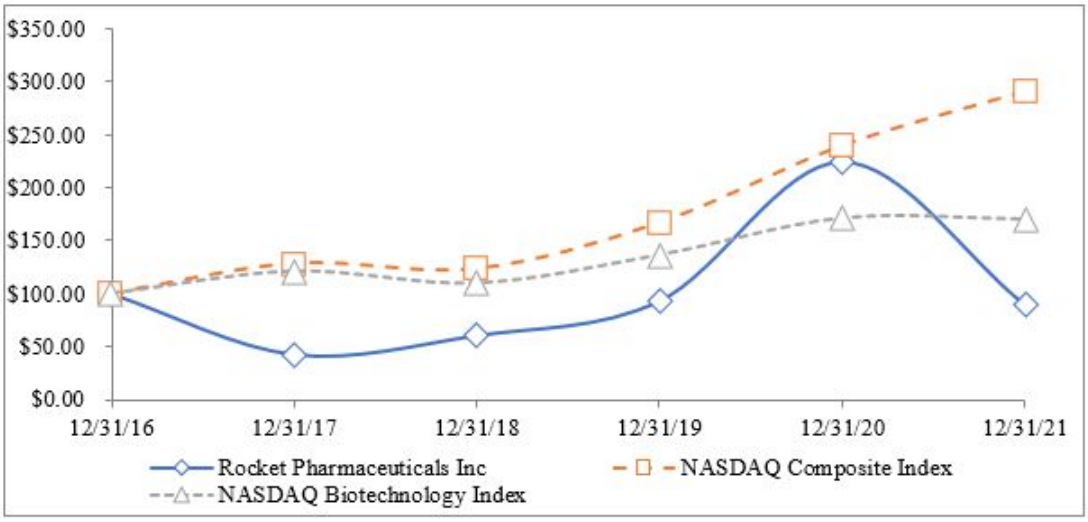
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities Market Information

Our common stock is traded on the Nasdaq Global Market under the symbol "RCKT" and on the Nasdaq Biotechnology Index (Nasdaq: NBI). On February 22, 2022, the last reported sale price for our common stock on the Nasdaq Global Market was $17.91 per share.

Stock Performance Graph

The graph set forth below compares the cumulative total stockholder return on our common stock between January 1, 2017 and December 31, 2021 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, 2017 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, 2022, there were 15 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers. We completed a reverse merger which closed on January 4, 2018. The stock performance information prior to January 4, 2018, represents the share price of our predecessor company Inotek Pharmaceuticals Corporation prior to the reverse merger as adjusted for a 4 for 1 reverse split completed upon the reverse merger.

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. In addition, the terms of our outstanding indebtedness restrict our ability to pay cash dividends, and any future indebtedness that we may incur could preclude us from paying cash dividends. Investors should not purchase our common stock with the expectation of receiving cash dividends.

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

There were no repurchases of our common stock during the year ended December 31, 2021.

Item 6. Reserved

Not applicable.

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 the terms the "combined company," "Rocket," the "Company," "we," "our" and "us" refer to Rocket Pharmaceuticals, Inc. (formerly known as Inotek Pharmaceuticals Corporation) and its subsidiaries. The term "Rocket Ltd" refers to privately-held Rocket Pharmaceuticals, Ltd. prior to its merger with Rome Merger Sub, a wholly owned subsidiary of Rocket Pharmaceuticals, Inc. The term "Inotek" refers to Inotek Pharmaceuticals Corporation and its subsidiaries prior to the reverse merger. For accounting purposes, the reverse merger is treated as a "reverse acquisition" under U.S. GAAP and Rocket Ltd is considered the accounting acquirer. Accordingly, the historical financial information included in this Annual Report, unless otherwise indicated or as the context otherwise requires, is that of Rocket Ltd prior to the reverse merger.

Management's Discussion and Analysis of Financial Condition and Results of Operations ("MD&A") is designed to provide a reader of Rocket's financial statements with a narrative from the perspective of management. Our management's discussion and analysis is intended to help the reader understand our results of operations and financial condition and is provided as an addition to, and should be read in connection with, our consolidated financial statements and the accompanying notes included elsewhere in this Annual Report on Form 10-K.

Introduction

We are a clinical-stage, multi-platform 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 ("LVV") programs. These 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 rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in potentially registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., we have a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

Recent Developments

On April 26, 2021, we redeemed in full our 2022 Convertible Notes prior to the redemption date. Holders of approximately $38.4 million remaining principal amount of the 2022 Convertible Notes converted such notes into approximately 1.3 million shares of the Company's common stock and cash in lieu of fractional shares. As of December 31, 2021, there were no 2022 Convertible Notes outstanding.

On August 2, 2021, holders of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 shares of common stock. As of December 31, 2021, there were no 2021 Convertible Notes outstanding.

On August 27, 2021, we entered into a Securities Purchase Agreement (the "Purchase Agreement") with a fund affiliated with RTW Investments, LP, our largest shareholder (the "Purchaser"), pursuant to which we agreed to sell and issue to the Purchaser, in a private placement (the "Private Placement"), 812,516 shares of our common stock at a purchase price of $32.48 per share for aggregate net proceeds of approximately $26.4 million after deducting estimated offering expenses payable. The Private Placement closed on August 31, 2021.

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, 2021, we raised net cash proceeds of approximately $680.5 million 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 that conduct R&D activities including, process development, preclinical, and clinical activities on our behalf;
- costs related to process development, 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;
- patent fees; 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 which 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.

Our direct R&D expenses consist principally of external costs, such as fees paid to investigators, consultants, laboratories and CROs in connection with our clinical studies, and costs related to acquiring and manufacturing clinical study materials. 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 of R&D expenses 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, 2021 and 2020. For the year ended December 31, 2019, we did not track expenses by program.

	For the Years Ended December 31,	
	2021	2020
Direct Expenses:		
Danon Disease (AAV) RP-A501	$ 15,804	$ 18,459
Leukocyte Adhesion Deficiency (LVV) RP-L201	24,222	5,531
Fanconi Anemia (LVV) RP-L102	15,453	15,015
Pyruvate Kinase Deficiency (LVV) RP-L301	4,206	4,990
Infantile Malignant Osteopetrosis (LVV) RP-L401 (1)	2,236	2,057
Other product candidates	4,576	1,321
Total direct expenses	66,496	47,373
Unallocated Expenses		
Employee compensation	20,780	14,137
Non-cash R&D expense related to the issuance of warrants	12,781	26,562
Stock based compensation expense	11,954	7,121
Depreciation and amortization expense	5,130	2,770
Laboratory and related expenses	3,359	4,240
Legal and patent fees	15	96
Professional Fees	1,797	1,443
Other expenses	3,164	1,696
Total other research and development expenses	58,979	58,066
Total research and development expense	$ 125,476	$ 105,438

(1) Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program is to be returned to academic innovators.

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 as well as any clinical studies and other R&D activities that we undertake;
- 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 in future periods for the foreseeable future as we seek to complete 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 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. 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 is related to the 2021 Convertible Notes, which were converted into common stock on August 2, 2021, the 2022 Convertible Notes, which were redeemed and converted into common stock in April 2021, and our financing lease obligation for the Cranbury, NJ facility.

Interest Income

Interest income is related to interest earned from investments and cash equivalents.

Results of Operations

Comparison of the Years Ended December 31, 2021 and 2020

The following table summarizes our results of operations for the years ended December 31, 2021 and 2020:

	For the Years Ended December 31,		
	2021	2020	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 125,476	$ 105,438	$ 20,038
General and administrative	41,772	28,865	12,907
Total operating expenses	167,248	134,303	32,945
Loss from operations	(167,248)	(134,303)	(32,945)
Research and development incentives	1,000	-	1,000
Interest expense	(2,977)	(6,967)	3,990
Interest and other income, net	3,068	2,150	918
(Amortization of premium) accretion of discount on investments - net	(2,912)	(580)	(2,332)
Total other expense, net	(1,821)	(5,397)	3,576
Net loss	$ (169,069)	$ (139,700)	$ (29,369)

Research and Development Expenses

R&D expenses increased $20.0 million to $125.5 million for the year ended December 31, 2021 compared to the year ended December 31, 2020. The increase was primarily due to an increase in manufacturing and development costs of $14.8 million, an increase in compensation and benefits of $6.6 million due to increased R&D headcount, an increase in non-cash stock compensation expense of $4.8 million, an increase in lab supplies and office expense of $2.9 million, an increase in depreciation and amortization of $1.9 million, offset by a decrease in new research agreements of $13.8 million in non-cash expenses due to the issuance of warrants in December 2020 and August and December 2021.

General and Administrative Expenses

G&A expenses increased $12.9 million to $41.8 million for the year ended December 31, 2021 compared to the year ended December 31, 2020. The increases in G&A expenses were primarily driven by an increase in non-cash stock compensation expense of $5.8 million, an increase in compensation and benefits of $1.8 million due to increased G&A headcount, an increase in office and administrative costs of $2.5 million due to increased insurance costs, an increase in commercial preparation expenses of $2.2 million, offset by a decrease in debt conversion expense recorded for the year ended December 31, 2020 of $2.0 million due to the refinancing of the 2021 Convertible Notes in February 2020. There were no debt conversion expenses recorded for the year ended December 31, 2021.

Other Expense, Net

Other expense, net was $1.8 million for the year ended December 31, 2021 compared to $5.4 million for the year ended December 31, 2020. The change was primarily due to reduced interest expense associated with the 2022 Convertible Notes which were fully redeemed in April 2021 and the 2021 Convertible Notes which were converted in August 2021, as well as a decrease of $2.3 million in accretion income related to our investments, due to lower interest rates for the year ended December 31, 2021 as compared to the year ended December 31, 2020.

Comparison of the Years Ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019:

	For the Years Ended December 31,		
	2020	**2019**	**Change**
		(in thousands)	
Operating expenses:			
Research and development	$ 105,438	$ 57,907	$ 47,531
General and administrative	28,865	18,244	10,621
Total operating expenses	134,303	76,151	58,152
Loss from operations	(134,303)	(76,151)	(58,152)
Research and development incentives	-	250	(250)
Interest expense	(6,967)	(5,958)	(1,009)
Interest and other income, net	2,150	3,414	(1,264)
(Amortization of premium) accretion of discount on investments - net	(580)	1,175	(1,755)
Total other expense, net	(5,397)	(1,119)	(4,278)
Net loss	$ (139,700)	$ (77,270)	$ (62,430)

Research and Development Expenses

R&D expenses increased $47.5 million to $105.4 million for the year ended December 31, 2020 compared to the year ended December 31, 2019. The increase was primarily the result of an increase in new research agreements totaling $26.6 million in non-cash R&D expenses due to the issuance of a warrant in December 2020, an increase in manufacturing consumables of $5.5 million, an increase in compensation expense of $6.2 million due to increased R&D headcount; an increase in clinical trials expense of $3.9 million, an increase in amortization expense related to the Cranbury, NJ lease of $1.7 million, and an increase in lab supplies of $2.0 million, offset by the CIRM grant of $1.1 million which was offset against LAD-1 R&D expenses, and a decrease in research agreements of $0.6 million primarily due to the $1.4 million license payment for the Stanford University's Laboratory for Cell and Gene Therapy ("LCGM") facility made in 2019.

General and Administrative Expenses

G&A expenses increased $10.6 million to $28.9 million for the year ended December 31, 2020 compared to the year ended December 31, 2019. The increase in G&A expenses was primarily driven by fees of $2.0 million incurred in connection with the February and June 2020 convertible notes exchange, an increase in non-cash stock compensation expense of $4.2 million, an increase in compensation and benefits of $2.5 million due to increased G&A headcount and an increase in office and administrative expense of $1.0 million due primarily to increased insurance expense for the year ended December 31, 2020 as compared to 2019.

Other Expense, Net

Other expense, net was $5.4 million for the year ended December 31, 2020 compared to other expense, net of $1.1 million for the year ended December 31, 2019. The change was primarily due to a decrease in interest income related to our investments of $2.8 million due to lower interest rates, an increase in interest expense of $1.0 million, and a decrease in research and development incentives due to the receipt of the New York City biotech tax credit in 2019, which was phased out by New York City and did not reoccur in 2020.

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, 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 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 $169.1 million, $139.7 million, and $77.3 million for the years ended December 31, 2021, 2020 and 2019, respectively. We have experienced negative cash flows from operations and has an accumulated deficit of $491.9 million as of December 31, 2021. As of December 31, 2021, we had $388.7 million of cash, cash equivalents and investments. We expect such resources would be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2023. We have funded our operations primarily through the sale of its equity and debt securities.

On April 30, 2019, the California Institute for Regenerative Medicine ("CIRM") awarded us up to $6.5 million under a CLIN2 grant award to support the clinical development of gene therapy for LAD-I. 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, University of California, Los Angeles ("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. In 2019, we received the first two grants from CIRM in the aggregate of $1.2 million which were included as an offset against R&D expenses. In 2020, we met additional CIRM milestones and received an additional $1.1 million milestone which was recorded as a reduction of R&D expenses in 2020. We received the additional milestone payments of $1.1 million and $1.0 million in January and April of 2021, respectively. As of December 31, 2021 we did not meet the next milestone and therefore no receivable has been recorded.

On November 12, 2020, the CIRM awarded us up to $3.7 million under a CLIN2 grant award to support the clinical development of our lentiviral vector (LVV)-based gene therapy, RP-L401, for the treatment of IMO. We received $1.0 million pursuant to the grant on January 4, 2021 related to the CIRM IMO award and recorded a receivable, included in prepaid and other assets in the consolidated balance sheet, and a reduction of R&D expenses of $0.9 million as of December 31, 2020. We recorded a reduction of research and development expense of $0.1 million for the year ended December 31, 2021. As of December 31, 2021, we did not meet the next milestone and no receivable has been recorded.

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.

Convertible Notes Payable

On January 4, 2018, in connection with the reverse merger with Inotek, Inotek's obligations under its outstanding convertible notes, with an aggregate principal value of $52.0 million, were assumed by us (the "2021 Convertible Notes"). The 2021 Convertible Notes were issued in 2016 and matured on August 1, 2021 ("Maturity Date"). The 2021 Convertible Notes were unsecured and accrued interest at a rate of 5.75% per annum. Each holder of a 2021 Convertible Note (the "Holder") had the option until the close of business on the second business day immediately preceding the Maturity Date to convert all, or any portion, of the 2021 Convertible Notes held by them at a conversion rate of 31.1876 shares of our common stock per $1.00 principal amount of 2021 Convertible Notes (the "Conversion Rate") which is $32.08 per share.

On February 20, 2020 and June 5, 2020, we entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of the 2021 Convertible Notes. Pursuant to the Exchange Agreements, on February 20, 2020, we exchanged approximately $39.35 million aggregate principal amount of the 2021 Convertible Notes (representing approximately 76% of the aggregate outstanding principal amount of the 2021 Convertible Notes) for (a) approximately $39.35 million aggregate principal amount of 6.25% Convertible Senior Notes due August 2022 (the "2022 Convertible Notes") (an exchange ratio equal to 1.00 2022 Convertible Note per exchanged 2021 Convertible Note) and (b) $0.1 million to pay the accrued and unpaid interest on the exchanged 2021 Convertible Notes from February 1, 2020, to February 20, 2020, the closing date of the February 20, 2020 exchange transactions. Additionally, we repurchased 3,000 shares of our common stock that have been retired for an aggregate amount of $72 from certain holders of the 2021 Convertible Notes participating in the exchange transactions in privately negotiated, private transactions.

Also pursuant to the Exchange Agreements, on June 12, 2020, we exchanged $7.5 million aggregate principal amount of the 2021 Convertible Notes for (a) $7.5 million aggregate principal amount of our newly issued 2022 Convertible Notes (an exchange ratio equal to 1.00 2022 Convertible Notes per exchanged 2021 Convertible Notes) and (b) approximately $11 to pay the accrued and unpaid interest on the exchanged 2021 Convertible Notes from, and including, February 1, 2020, to, but excluding, the closing date of the exchange transaction, adjusted to take into account the unearned accrued interest on the 2022 Convertible Notes from, and including, February 20, 2020, to, but excluding, the closing date of the exchange transaction.

In December, 2020, $8.5 million principal amount, representing a carrying value of $7.6 million of the 2022 Convertible Notes were converted into 298,562 shares of common stock.

On April 26, 2021, we called for the redemption of the remaining $38.4 million principal amount of the 2022 Convertible Notes as our stock price traded above the Conversion Rate for at least 20 trading days during a 30-day consecutive trading period. On April 26, 2021, we redeemed in full the 2022 Convertible Notes prior to the redemption date. Holders of approximately $38.4 million remaining principal amount of the 2022 Convertible Notes converted such notes in accordance with the terms of the Exchange Agreements into approximately 1.3 million shares of our common stock and cash in lieu of fractional shares. As of December 31, 2021, there were no 2022 Convertible Notes outstanding.

On August 2, 2021, holders of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 shares of our common stock. As of December 31, 2021, there were no 2021 Convertible Notes outstanding.

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 “Item 8. Financial Statements”, “Note 11. Income Taxes” and “Note 12. Commitments and Contingencies”, respectively.

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 for each of the periods presented:

	For the Years Ended December 31,		
	2021	**2020**	**2019**
Cash used in operating activities	$ (121,163)	$ (74,640)	$ (64,663)
Cash provided by (used in) investing activities	18,853	(96,591)	(39,011)
Cash provided by financing activities	37,681	282,989	177,791
Net change in cash, cash equivalents and restricted cash	$ (64,629)	$ 111,758	$ 74,117

Net Cash Used in Operating Activities

During the year ended December 31, 2021, operating activities used $121.2 million of cash, 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, accretion of discount 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.

During the year ended December 31, 2020, operating activities used $74.6 million of cash, primarily resulting from our net loss of $139.7 million and net changes in our operating assets and liabilities of $15.0 million, partially offset by net non-cash charges of $50.0 million, including expenses in connection with the issuance of warrants of $26.6 million, stock-based compensation expense of $18.6 million and accretion of discount on convertible notes of $2.8 million. Changes in our operating assets and liabilities for the year ended December 31, 2020 consisted of an increase in finance lease liability of $5.6 million, an increase in accounts payable and accrued expenses of $6.4 million and an increase in prepaid expenses and other assets of $1.5 million.

During the year ended December 31, 2019, operating activities used $64.7 million of cash, primarily resulting from our net loss of $77.3 million and net changes in our operating assets and liabilities of $3.7 million, partially offset by net non-cash charges of $16.3 million, including stock-based compensation expense of $13.4 million and accretion of discount on convertible notes of $3.6 million. Changes in our operating assets and liabilities for the year ended December 31, 2019 consisted of a decrease in accounts payable and accrued expenses of $2.7 million and an increase in prepaid expenses and other assets of $0.9 million.

Net Cash Provided by (Used in) Investing Activities

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.

During the year ended December 31, 2020, net cash used in investing activities was $96.6 million, consisting of proceeds of $141.8 million from the maturities of investments, offset by purchases of investments of $209.3 million, purchases of property and equipment of $20.6 million, and payments made to acquire right of use asset of $8.5 million.

During the year ended December 31, 2019, net cash used in investing activities was $39.0 million, consisting primarily of purchases of property and equipment which is considered construction in progress of $23.3 million related primarily to our Cranbury, New Jersey facility, and investment purchases of $184.3 million offset by proceeds of $168.6 million from the maturities of investments.

Net Cash Provided by Financing Activities

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 the August 2021 Private Placement and issuance of common stock, pursuant to exercises of stock options.

During the year ended December 31, 2020, net cash provided by financing activities was $283.0 million, consisting primarily of proceeds from the issuance of common stock for $280.8 million. On December 14, 2020, we completed a public offering of 5,339,286 shares of common stock, which included the full exercise of the underwriters' option to purchase an additional 696,428 shares of our common stock, at a public offering price of $56.00 per share. The net proceeds to Rocket from the December 2020 public offering were approximately $280.8 million.

During the year ended December 31, 2019, net cash provided by financing activities was $177.8 million, consisting of proceeds from the issuance of common stock for $177.8 million. On April 18, 2019, we completed a public offering of 5,175,000 shares of common stock. The net proceeds to Rocket from the April 2019 public offering were approximately $86.1 million. On December 10, 2019, we completed a public offering of 4,393,000 shares of common stock. The net proceeds to Rocket from the December 2019 public offering were approximately $91.7 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 in conformity with accounting principles generally accepted in the United States ("US 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 Research and Development Expenses.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development 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 research and development 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 nonemployee 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 statement 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

In June 2016, the FASB issued ASU 2016-13, Financial Instruments – Credit Losses (Topic 362): Measurement of Credit Losses on Financial Statements ("ASU 2016-13"). The new standard requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. It also limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The targeted transition relief standard allows filers an option to irrevocably elect the fair value option of ASC 825-10, Financial Instruments-Overall, applied on an instrument-by-instrument basis for eligible instruments. The new standard was effective beginning January 1, 2021. The adoption of ASU 2016-13, and related updates, did not have a material impact on the Company's consolidated financial position and results of operations.

Item 7A. Quantitative and Qualitative Disclosures about Market Risks

Not Applicable

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, 2021, our principal executive officer and principal financial and accounting officers 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(e) and 15d-15(e) 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 principal financial and accounting officers, 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, 2021, 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, 2021, based on criteria established in the COSO 2013 framework.

The effectiveness of our internal control over financial reporting as of December 31, 2021 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

None.

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 2022 Annual Meeting of Stockholders ("Proxy Statement") under the headings "Election of Directors," "Executive Officers," "Delinquent Section 16(a) Reports" 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 "Executive Compensation" and "Director Compensation" 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 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 “Securities Authorized for Issuance Under Equity Compensation Plans” 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 (Filed as Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 13, 2017, and incorporated herein by reference)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (Filed as Exhibit 3.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 31, 2015, and incorporated herein by reference)
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 (Filed as Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (Filed as Exhibit 3.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference)
3.4	Certificate of Amendment to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective June 25, 2018 (Filed as Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2019, and incorporated herein by reference)
3.5	Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of March 29, 2018 (Filed as Exhibit 3.4 to the Company's registration statement on Form 8-A/A, as amended (001-36829), filed with the SEC on January 11, 2018, and incorporated herein by reference)
4.1	Form of Common Stock Certificate of Rocket Pharmaceuticals, Inc. (Filed as Exhibit 4.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference)
4.2	Description of Securities (Filed as Exhibit 4.8 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.1#	2004 Stock Option and Incentive Plan (Filed as Exhibit 10.1 to the Company's Registration Statement on Form S-1 (333-199859), filed with the SEC on November 5, 2014, as amended, and incorporated herein by reference)
10.2#	Rocket Pharmaceuticals, Inc. Second Amended and Restated 2014 Stock Option and Incentive Plan (Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2018, and incorporated herein by reference)
10.3#	Form of Incentive Stock Option Agreement (Employees) (Filed as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, and incorporated herein by reference)
10.4#	Form of Non-Qualified Stock Option Agreement (Employees) (Filed as Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, and incorporated herein by reference)
10.5#	Form of Non-Qualified Stock Option Agreement (Non-Employee Directors) (Filed as Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, and incorporated herein by reference)
10.6#	Form of Non-Qualified Stock Option Agreement (Consultants) (Filed as Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, and incorporated herein by reference)
10.6.1#	Form of Restricted Stock Unit Award Agreement (Filed as Exhibit 10.6.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.7#	Rocket Pharmaceuticals, Ltd. 2015 Share Option Plan (Filed as Exhibit 10.3 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 7, 2018, and incorporated herein by reference)
10.8#	Letter Agreement, dated as of July 28, 2014, by and between the Registrant and David P. Southwell (Filed as Exhibit 10.3 to the Company's Registration Statement on Form S-1 (333-199859), filed with the SEC on November 5, 2014, as amended, and incorporated herein by reference)
10.9#	Amendment to Offer Letter, effective as of September 1, 2017, by and between Inotek and David Southwell (Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 1, 2017, and incorporated herein by reference)
10.10#*	Offer Letter, dated September 29, 2017, by and between the Registrant and Raj Prabhakar.
10.11#	Offer Letter, dated November 25, 2020, by and between the registrant and Carlos Garcia-Parada. (Filed as Exhibit 10.11 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.12#*	Offer Letter, dated April 29, 2021, by and between the registrant and Isabel Carmona.
10.13**	Amended and Restated Lease Agreement, dated as of June 26, 2019, by and between Rocket Pharmaceuticals, Inc. and Cedar Brook 12 Corporate Center, L.P. (Filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 8, 2018, and incorporated herein by reference)
10.14#	Rocket Pharmaceuticals, Inc. Amended and Restated 2014 Employee Stock Purchase Plan (Filed as Exhibit 10.10 to the Company's Annual Report on Form 8-K (001-36829), filed with the SEC on March 7, 2018, and incorporated herein by reference)
10.15#	Form of Indemnification Agreement, to be entered into between the Registrant and its directors (Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference)
10.16#	Form of Indemnification Agreement, to be entered into between the Registrant and its officers (Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference)
10.17#	Form of Severance and Change of Control Agreements, to be entered into between the Registrant and certain of its officers (Filed as Exhibit 10.16 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)

10.20	Agreement of Lease, dated as of June 6, 2018, by and between Rocket Pharmaceuticals, Inc. and ESRT Empire State Building, L.L.C., (Filed as Exhibit 10.6 to the Company's Quarterly Report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, and incorporated herein by reference)
10.22†	License Agreement, dated as of November 19, 2018, by and between Rocket Pharmaceuticals, Ltd. and REGENXBIO Inc. (Filed as Exhibit 10.26 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 8, 2019, and incorporated herein by reference)
10.23	Warrant to Purchase Shares of Common Stock, dated as of December 21, 2020, by and between the Registrant and Neptune Consulting, LLC. (Filed as Exhibit 10.23 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.24	Warrant to Purchase Shares of Series Preferred Stock, dated as of June 28, 2013, by and between Inotek Pharmaceuticals Corporation and Horizon Technology Finance Corporation (Filed as Exhibit 10.24 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.25	Warrant to Purchase Shares of Series Preferred Stock dated as of June 28, 2013, by and between Inotek Pharmaceuticals Corporation and Fortress Credit Co LLC (Filed as Exhibit 10.25 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 1, 2021, and incorporated herein by reference)
10.26*	Warrant to Purchase Shares of Common Stock, dated as of December 17, 2021, by and between the Registrant and Neptune Consulting, LLC. (First Indication)
10.27*	Warrant to Purchase Shares of Common Stock, dated as of December 17, 2021, by and between the Registrant and Neptune Consulting, LLC. (Second Indication)
10.28	Securities Purchase Agreement, dated as of August 27, 2021, by and among Rocket Pharmaceuticals, Inc., and each of those persons listed as a Purchaser on the Schedule of Purchasers attached as Schedule I thereto (Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on August 30, 2021, and incorporated herein by reference)
10.29	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 (Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on August 30, 2021, and incorporated herein by reference).
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
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

101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Link Document.
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 Section 13 or 15(d) of the Securities Exchange Act of 1934, Rocket Pharmaceuticals, Inc. (the Registrant) has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Cranbury, State of New Jersey, on February 28, 2022.

Rocket Pharmaceuticals, Inc.

By: /s/ Gaurav Shah, MD
Gaurav Shah, MD
President and Chief Executive Officer

POWER OF ATTORNEY

Each person whose individual signature appears below hereby constitutes and appoints Gaurav Shah, MD and Carlos Garcia-Parada, 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 28, 2022
/s/ Carlos Garcia-Parada, MBA Carlos Garcia-Parada	Chief Financial Officer *(Principal Financial Officer)*	February 28, 2022
/s/ John C. Militello John C. Militello	VP, Finance, Senior Controller & Treasurer *(Principal Accounting Officer)*	February 28, 2022
/s/ Carsten Boess Carsten Boess	Director	February 28, 2022
/s/ Pedro Granadillo Pedro Granadillo	Director	February 28, 2022
/s/ Gotham Makker, MD Gotham Makker, MD	Director	February 28, 2022
/s/ David P. Southwell David P. Southwell	Director	February 28, 2022
/s/ Roderick Wong, MD Roderick Wong, MD	Director	February 28, 2022
/s/ Naveen Yalamanchi, MD Naveen Yalamanchi, MD	Director	February 28, 2022
/s/ Elisabeth Björk Elisabeth Björk	Director	February 28, 2022

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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, 2021 and 2020, and the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2021, 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, 2021 and 2020, and the consolidated results of its their operations and their cash flows for each of the years in the three-year period ended December 31, 2021, 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, 2021, 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 28, 2022 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 Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. 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 matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Accruals for research and development expenses

As disclosed in Note 3 to the consolidated 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 accrued research and development expenses as of December 31, 2021 were approximately $12 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 consolidated 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 total payments made by the Company and the amount owed by the Company as of December 31, 2021 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 to the resulting accruals.

/s/ EisnerAmper LLP

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

EISNERAMPER LLP
Iselin, New Jersey
February 28, 2022

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders
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, 2021, 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, 2021 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 the Company as of December 31, 2021 and 2020, and the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2021, and the related notes and our report dated February 28, 2022 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 28, 2022

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

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 232,694	$ 297,098
Investments	156,046	185,621
Prepaid expenses and other current assets	3,319	4,626
Total current assets	392,059	487,345
Property and equipment, net	22,299	19,206
Goodwill	30,815	30,815
Restricted cash	1,343	1,568
Deposits	455	455
Operating lease right-of-use assets	1,569	914
Finance lease right-of-use asset	48,480	50,521
Total assets	$ 497,020	$ 590,824
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 19,615	$ 25,472
Convertible notes, net of unamortized discount, current	-	4,875
Operating lease liabilities, current	863	626
Finance lease liability, current	1,689	1,644
Total current liabilities	22,167	32,617
Convertible notes, net of unamortized discount, non-current	-	35,066
Operating lease liabilities, non-current	905	498
Finance lease liability, non-current	19,144	18,988
Other liabilities	80	136
Total liabilities	42,296	87,305
Commitments and contingencies (Note 12)		
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; 64,505,889 and 60,996,367 shares issued and outstanding at December 31, 2021 and 2020, respectively	645	610
Additional paid-in capital	946,152	825,794
Accumulated other comprehensive loss	(161)	(42)
Accumulated deficit	(491,912)	(322,843)
Total stockholders' equity	454,724	503,519
Total liabilities and stockholders' equity	$ 497,020	$ 590,824

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,		
	2021	**2020**	**2019**
Revenue	$ -	$ -	$ -
Operating expenses:			
Research and development	125,476	105,438	57,907
General and administrative	41,772	28,865	18,244
Total operating expenses	167,248	134,303	76,151
Loss from operations	(167,248)	(134,303)	(76,151)
Research and development incentives	1,000	-	250
Interest expense	(2,977)	(6,967)	(5,958)
Interest and other income, net	3,068	2,150	3,414
(Amortization of premium) accretion of discount on investments - net	(2,912)	(580)	1,175
Net loss	$ (169,069)	$ (139,700)	$ (77,270)
Net loss per share attributable to common stockholders - basic and diluted	$ (2.67)	$ (2.52)	$ (1.58)
Weighted-average common shares outstanding - basic and diluted	63,235,417	55,380,740	49,010,358

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,		
	2021	**2020**	**2019**
Net loss	$ (169,069)	$ (139,700)	$ (77,270)
Other comprehensive loss			
Net unrealized (loss) gain on investments	(119)	(62)	147
Total comprehensive loss	$ (169,188)	$ (139,762)	$ (77,123)

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						
	Shares	Amount	Treasury Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2018	45,194,736	$ 452	$ (668)	$ 300,253	$ (127)	$ (105,873)	$ 194,037
Issuance of common stock, net of issuance costs	9,568,000	96	-	177,664	-	-	177,760
Issuance of common stock pursuant to exercise of stock options	110,325	1	-	30	-	-	31
Treasury stock purchases	-	-	(725)	(1)	-	-	(726)
Issuance of treasury stock pursuant to exercise of stock options	-	-	(397)	-	-	-	(397)
Retirement of treasury stock	(100,000)	(1)	1,393	(1,392)	-	-	-
Sale of treasury stock	-	-	344	-	-	-	344
Unrealized comprehensive gain on investments	-	-	-	-	147	-	147
Share-based compensation	-	-	-	13,371	-	-	13,371
Net loss	-	-	-	-	-	(77,270)	(77,270)
Balance at December 31, 2019	54,773,061	548	(53)	489,925	20	(183,143)	307,297
Issuance of common stock, net of issuance costs	5,339,286	53	-	280,710	-	-	280,763
Issuance of common stock pursuant to exercise of stock options	586,857	6	-	2,552	-	-	2,558
Issuance of common stock pursuant to exercise of warrant	1,601	-	-	-	-	-	-
Issuance of common stock pursuant to conversion of notes	298,562	3	-	7,626	-	-	7,629
Issuance of warrants	-	-	-	26,562	-	-	26,562
Stock repurchase	(3,000)	-	-	(72)	-	-	(72)
Sale of treasury stock	-	-	667	(76)	-	-	591
Issuance of treasury stock pursuant to exercise of stock options	-	-	(614)	-	-	-	(614)
Unrealized comprehensive loss on marketable securities	-	-	-	-	(62)	-	(62)
Share-based compensation	-	-	-	18,567	-	-	18,567
Net loss	-	-	-	-	-	(139,700)	(139,700)
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)
Share-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

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,		
	2021	**2020**	**2019**
Operating Activities:			
Net loss	$ (169,069)	$ (139,700)	$ (77,270)
Adjustments to reconcile net loss to net cash used in operating activities:			
Accretion of discount on convertible notes	753	2,758	3,602
Depreciation and amortization of property and equipment	3,240	1,145	426
Amortization of right of use asset	2,133	5,105	-
Write down of property and equipment, net	261	419	-
Stock-based compensation	29,237	18,567	13,371
Expense in connection with warrant issuances	12,781	26,562	-
Accretion of discount (amortization of premium) on investments, net	2,887	580	(1,066)
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	1,307	(1,517)	(917)
Accounts payable and accrued expenses	(4,827)	11,015	(2,722)
Operating lease liabilities	(11)	(139)	(87)
Finance lease liability	201	452	-
Other long term liabilities	(56)	113	-
Net cash used in operating activities	(121,163)	(74,640)	(64,663)
Investing activities:			
Purchases of investments	(245,875)	(209,343)	(184,298)
Proceeds from maturities of investments	272,443	141,811	168,556
Payments made to acquire right of use asset	(95)	(8,452)	-
Purchases of property and equipment	(7,620)	(20,607)	(23,269)
Net cash provided by (used in) investing activities	18,853	(96,591)	(39,011)
Financing activities:			
Issuance of common stock, net of issuance costs	26,354	280,763	177,760
Issuance of common stock, pursuant to exercise of stock options	11,327	2,558	31
Common stock repurchase	-	(72)	-
Proceeds from sale of treasury stock	-	591	344
Payment of withholding tax on option exercises	-	(614)	(344)
Convertible notes refinancing costs to the lender	-	(237)	-
Net cash provided by financing activities	37,681	282,989	177,791
Net change in cash, cash equivalents and restricted cash	(64,629)	111,758	74,117
Cash, cash equivalents and restricted cash at beginning of period	298,666	186,908	112,791
Cash, cash equivalents and restricted cash at end of period	$ 234,037	$ 298,666	$ 186,908
Supplemental disclosure of non-cash financing and investing activities:			
Accrued purchases of property and equipment	$ 728	$ 1,756	$ 4,650
Retirement of treasury stock	$ -	$ -	$ 1,393
Treasury stock purchases paid in prior year	$ -	$ -	$ 726
Withholding tax payable on shares withheld in treasury stock	$ -	$ -	$ 53
Unrealized (loss) gain on investments	$ (119)	$ (62)	$ 147
Conversion of 2021 and 2022 convertible notes into common stock	$ 40,694	$ 7,629	$ -
Finance lease right of use asset and lease liability	$ -	$ 20,179	$ -
Reclassification of construction in process to finance right of use asset	$ 98	$ 26,465	$ -
Supplemental cash flow information:			
Cash paid for interest	$ 148	$ 2,960	$ 2,990
Cash paid for income taxes	$ -	$ -	$ 26

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

Rocket Pharmaceuticals, Inc.
Notes to Consolidated Financial Statements
(in thousands, except share and per share amounts)

1. Nature of Business and Basis of Presentation

Rocket Pharmaceuticals, Inc. ("Rocket" or the "Company") is a clinical-stage, multi-platform biotechnology company focused on the development of gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. Rocket has three clinical-stage *ex vivo* lentiviral vector ("LVV") programs. These 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 rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., Rocket has a clinical stage in vivo adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. Additional discovery efforts on a gene therapy program for the less common FA subtypes C and G is ongoing. The Company has global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, the Company made a decision to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program is to be returned to academic innovators. The Company has opted to focus available resources towards advancement of RP-A501, RP-L102, RP-L201 and RP-L301, based on the clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

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 $491.9 million as of December 31, 2021. As of December 31, 2021, the Company has $388.7 million of cash, cash equivalents and investments. During the year ended December 31, 2021, the Company issued and sold 812,516 shares of its common stock at a purchase price of $32.48 per share for aggregate net proceeds of approximately $26.4 million in a private placement transaction to a fund affiliated with RTW Investments, LP., the Company's largest shareholder (see Note 15). The Company expects such resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements into the second half of 2023.

In April 2021, the Company called for the redemption of the remaining $38.4 million principal balance of the Company's 6.25%, 2022 Convertible Senior Notes due 2022 which were converted into common stock. On August 2, 2021, holders of $5.15 million of the 2021 Convertible Notes converted the remaining $5.15 million remaining balance of the 2021 Convertible Notes into common stock (see Note 7). As of December 31, 2021, none of the 2021 Convertible Notes or 2022 Convertible Notes were outstanding.

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 United States ("US GAAP"). All intercompany accounts have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with US 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 impairment, the accrual of research and development 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 12 "Commitments and Contingencies" 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, 2021	December 31, 2020
Cash and cash equivalents	$ 232,694	$ 297,098
Restricted cash	1,343	1,568
	$ 234,037	$ 298,666

Government Grants

Research and development expense is presented net of reimbursements from the California Institute for Regenerative Medicine ("CIRM"), which are recognized over the period necessary to match the reimbursement with the related costs when it is probable that the Company has complied with the CIRM conditions and will receive the reimbursement. During the years ended December 31, 2021, 2020, and 2019, the Company offset $0.1 million, $3.6 million and $1.2 million of CIRM grant funds against research and development ("R&D") expenses (See Note 14 "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, and Corporate, Government Municipal and Agency Bonds. The Company has adopted an investment policy that 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+/Aa1 rated, thereby reducing credit risk exposure.

Investments

Investments consist of investments in United States Treasury securities and Corporate, Municipal and Agency 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. There were no realized gains or losses on investments for the years ended December 31, 2021, 2020 and 2019. For the years ended December 31, 2021, 2020 and 2019, there was $0.1 million of net unrealized losses, $0.1 million of net unrealized losses and $0.1 million of net unrealized gains on investments, respectively.

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, 2021, 2020 and 2019.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. The estimated useful lives 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 for impairment whenever events or changes in circumstances indicate that the asset's carrying amount may not be recoverable. The Company conducted our long-lived asset impairment analyses in accordance with ASC 360-10-15, "Impairment or Disposal of Long-Lived Assets." ASC 360-10-15 requires us 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. There is no impairment of long-lived assets as of and for the years ended December 31, 2021, 2020 and 2019.

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* ("ASC 820") 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 these instruments.

Research and Development Expenses

R&D costs, which include salaries and staff costs, license costs, manufacturing and development costs, clinical trial 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 ("ASC 730"). The Company does not currently have any commercial biopharmaceutical products and does not expect to have any for several years, if at all. Accordingly, R&D costs are expensed as incurred. While certain of the Company's R&D costs may have future benefits, the policy of expensing all R&D expenditures is predicated on the fact that the Company has no history of successful commercialization of product candidates to base any estimate of the number of future periods that would be benefited.

Foreign Currency Transactions

Certain transactions during the years ended December 31, 2021, 2020 and 2019 are denominated in Euros and British pounds. Gains and losses on foreign currency transactions were not significant for the years ended December 31, 2021, 2020 and 2019.

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. 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 nonemployee 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 stock price, 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 statement 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.

NYS Life Sciences Research and Development Tax Credit

New York State allows investors and owners of emerging technology companies focused on biotechnology to claim a tax credit against their New York State Tax return for certain expenditures incurred in New York State, including applicable R&D related expenditures. The credit is recognized as research and development incentives when the eligibility and amount has been approved by New York State. During the years ended December 31, 2021, 2020 and 2019, the Company recorded research and development incentive income of $1.0 million, $0, and $0, respectively related to the NYS Life Sciences Research and Development Tax Credit.

NYC Biotechnology Tax Credit Program

New York City allowed investors and owners of emerging technology companies focused on biotechnology to claim a tax credit against the General Corporation Tax and Unincorporated Business Tax for amounts paid or incurred for certain facilities, operations, and employee training in New York City. The credit was recognized as research and development incentives when approved by New York City of the eligibility for the credit and the credit amount. During the years ended December 31, 2021, 2020 and 2019, the Company recorded research and development incentive income of $0 million, $0, and $0.3 million, respectively related to the NYC Biotechnology Program. This program was not renewed by NYC upon its expiration at the end of 2019.

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, 2021 and 2020.

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. 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.

Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation.

Recent Accounting Pronouncements

In June 2016, the FASB issued ASU 2016-13, Financial Instruments – Credit Losses (Topic 362): Measurement of Credit Losses on Financial Statements ("ASU 2016-13"). The new standard requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. It also limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The targeted transition relief standard allows filers an option to irrevocably elect the fair value option of ASC 825-10, Financial Instruments-Overall, applied on an instrument-by-instrument basis for eligible instruments. The new standard was effective beginning January 1, 2021. The adoption of ASU 2016-13, and related updates, did not have a material impact on the Company's consolidated financial position and results of operations.

There were no other recent accounting pronouncements that impacted the Company or are expected to have a significant effect on the 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, 2021 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 179,900	$ -	$ -	$ 179,900
	179,900	-	-	179,900
Investments:				
United States Treasury securities	44,045	-	-	44,045
Corporate Bonds	-	96,696	-	96,696
Municipal Bonds	-	6,000	-	6,000
Agency Bonds	-	9,305	-	9,305
	44,045	112,001	-	156,046
	$ 223,945	$ 112,001	$ -	$ 335,946

	Fair Value Measurements as of December 31, 2020 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 193,312	$ -	$ -	$ 193,312
United States Treasury securities	62,497	-	-	62,497
Corporate Bonds	-	501	-	501
Agency Bonds	-	8,015	-	8,015
	255,809	8,516	-	264,325
Investments:				
United States Treasury securities	112,328	-	-	112,328
Corporate Bonds	-	63,710	-	63,710
Municipal Bonds	-	6,000	-	6,000
Agency Bonds	-	3,583	-	3,583
	112,328	73,293	-	185,621
	$ 368,137	$ 81,809	$ -	$ 449,946

The Company classifies its money market mutual funds and U.S. Treasury securities 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 Corporate, Municipal 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.

5. Property and Equipment, Net

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

	December 31, 2021	December 31, 2020
Laboratory equipment	$ 12,600	$ 7,807
Machinery and equipment	10,432	9,933
Computer equipment	218	218
Furniture and fixtures	1,963	1,880
Leasehold improvements	407	29
Internal use software	1,902	1,385
	27,522	21,252
Less: accumulated depreciation and amortization	(5,223)	(2,046)
	$ 22,299	$ 19,206

Depreciation and amortization during the years ended December 31, 2021, 2020, and 2019 was $3.2 million, $1.1 million and $0.4 million, respectively.

6. Accounts Payable and Accrued Expenses

At December 31, 2021 and 2020, the Company's accounts payable and accrued expenses consisted of the following:

	December 31, 2021	December 31, 2020
Research and development	$ 12,082	$ 14,962
Property and equipment	725	1,756
Employee compensation	4,533	4,875
Accrued interest	-	1,122
Government grant payable	597	590
Professional fees	1,196	1,332
Other	482	835
	$ 19,615	$ 25,472

7. Convertible Notes Payable

2021 Convertible Notes

On January 4, 2018, in connection with its reverse merger with Inotek Pharmaceuticals, Corporation ("Inotek"), the Company assumed the obligations of Inotek under its outstanding convertible notes, with an aggregate original principal amount of $52.0 million, (the "2021 Convertible Notes"). The 2021 Convertible Notes were issued in 2016 and were initially scheduled to mature on August 1, 2021 (the "Maturity Date"). The 2021 Convertible Notes were unsecured and accrued interest at a rate of 5.75% per annum and interest was payable semi-annually on February 1 and August 1 of each year. Each holder of the 2021 Convertible Notes ("Holder") had the option until the close of business on the second business day immediately preceding the Maturity Date to convert all, or any portion, of the 2021 Convertible Notes held by it at a conversion rate of 31.1876 shares of the Company's common stock per $1.00 principal amount of 2021 Convertible Notes (the "Conversion Rate") which is $32.08 per share.

On August 2, 2021, holders of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 shares of the Company's common. As of December 31, 2021, none of the 2021 Convertible Notes were outstanding.

2022 Convertible Notes

On February 20, 2020, and June 5, 2020, the Company entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of the 2021 Convertible Notes. Pursuant to the Exchange Agreements, on February 20, 2020, the Company exchanged approximately $39.35 million aggregate principal amount of the 2021 Convertible Notes for (a) approximately $39.35 million aggregate principal amount of 6.25% Convertible Senior Notes due August 2022 (the "2022 Convertible Notes"). Also pursuant to the Exchange Agreements, on June 12, 2020, the Company exchanged $7.5 million aggregate principal amount of the 2021 Convertible Notes for (a) $7.5 million aggregate principal amount of its newly issued 6.25% Convertible Senior Notes due 2022.

The conversion rate for the 2022 Convertible Notes was initially 31.1876 shares of the Company's common stock per $1.00 principal amount of 2022 Convertible Notes, which was equivalent to an initial conversion price of approximately $32.06 per share of common stock. The Company may have redeemed for cash all or any portion of the 2022 Convertible Notes, at its option, if the last reported sale price of its common stock was equal to or greater than 130% of the conversion price then in effect for at least 20 trading days during any 30 consecutive trading day period.

In December 2020, $8.5 million principal amount, representing a carrying value of $7.6 million of the 2022 Convertible Notes was converted into 298,562 shares of the Company's common stock.

On April 26, 2021, the Company called for the redemption of the remaining $38.4 million principal amount of the 2022 Convertible Notes as the Company's stock price traded above the Conversion Rate for at least 20 trading days during a 30-day consecutive trading period. On April 26, 2021, the Company redeemed in full the 2022 Convertible Notes prior to the redemption date. Holders of approximately $38.4 million remaining principal amount of the 2022 Convertible Notes converted such notes in accordance with the terms of the Exchange Agreements into approximately 1.3 million shares of the Company's common stock and cash in lieu of fractional shares. In accordance with ASC 470-*Debt*, the settlement of the 2022 Convertible Notes is accounted for as a conversion since the 2022 Convertible Notes did not include a beneficial conversion feature and the carrying amount of the 2022 Convertible Notes.

The table below summarizes the carrying value of the 2021 and 2022 Convertible Notes as of December 31, 2021 and 2020:

	2021 Notes		2022 Notes	
	December 31, 2021	December 31, 2020	December 31, 2021	December 31, 2020
Principal amount	$ -	$ 5,150	$ -	$ 38,350
Discount	-	(275)	-	(3,284)
Carrying value	$ -	$ 4,875	$ -	$ 35,066

Accretion of the 2021 Convertible Notes discount was $0.3 million, $1.3 million, and $3.6 million for the years ended December 31, 2021, 2020, and 2019, respectively. Accretion of the 2022 Convertible Notes discount was $0.5 million and $1.5 million for the years ended December 31, 2021 and December 31, 2020.

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 fiscal 2019, the Company recorded treasury stock of $0.1 million for shares withheld to pay the payroll tax liability of an option exercise which was remitted in January 2020. There was no treasury stock as of December 31, 2021 and 2020.

Private Placement

On August 27, 2021, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with a fund affiliated with RTW Investments, LP, the Company's largest shareholder (the "Purchaser"), pursuant to which the Company agreed to sell and issue to the Purchaser, in a private placement (the "Private Placement"), 812,516 shares of the Company's common stock at a purchase price of $32.48 per share for aggregate net proceeds of approximately $26.4 million after deducting estimated offering expenses payable. The Private Placement closed on August 31, 2021.

Public Offerings

On December 14, 2020, the Company completed a public offering of 5,339,286 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 696,428 shares of our common stock, at a public offering price of $56.00 per share. The gross proceeds to Rocket from the public offering were approximately $299.0 million, net of $18.2 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $280.8 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:

	Years Ended December 31,		
	2021	2020	2019
Risk-free interest rate	0.83%	1.00%	2.26%
Expected term (in years)	5.84	5.84	5.81
Expected volatility	69.27%	76.98%	75.71%
Expected dividend yield	0.00%	0.00%	0.00%
Exercise price	$ 51.20	$ 22.96	$ 14.47
Fair value of common stock	$ 51.20	$ 22.96	$ 14.47

The following table summarizes stock option activity for the years ended December 31, 2021 and 2020, under the Revised 2014 Plan:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2019	9,763,541	$ 5.95	6.96	$ 164,021
Granted	2,193,546	22.96	8.97	
Exercised	(586,857)	4.82		13,494
Cancelled	(319,299)	15.82		
Outstanding as of December 31, 2020	11,050,931	$ 9.10	6.55	$ 504,079
Granted	1,671,759	51.20	8.58	
Exercised	(1,209,960)	9.32		54,487
Cancelled	(368,969)	35.87		
Outstanding as of December 31, 2021	11,143,761	$ 14.51	5.95	$ 128,817
Options vested and exercisable as of December 31, 2021	8,692,898	$ 7.31	5.11	$ 127,571
Options unvested as of December 31, 2021	2,450,863	$ 40.01	8.93	

The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2021, 2020 and 2019 was $31.07, $15.10, and $9.58, respectively.

The total fair value of options vested during the years ended December 31, 2021, 2020 and 2019 was $22.6 million, $15.6 million and $63.1 million, respectively.

Stock-Based Compensation

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

	Years Ended December 31,		
	2021	2020	2019
Stock options	$ 28,811	$ 18,527	$ 13,371
Restricted stock units	426	40	-
Total share based compensation expense	$ 29,237	$ 18,567	$ 13,371

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

	Years Ended December 31,		
	2021	2020	2019
Research and development	$ 11,954	$ 7,355	$ 6,153
General and administrative	17,283	11,212	7,218
Total share based compensation expense	$ 29,237	$ 18,567	$ 13,371

As of December 31, 2021 and 2020, the Company had an aggregate of $46.3 million and $30.4 million of unrecognized stock-based compensation expense, which is expected to be recognized over the weighted average period of 1.8 years and 2.1 years, respectively.

Restricted Stock Units ("RSU")

The following table summarizes our RSU activity for the years ended December 31, 2021 and 2020:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2019	-	$ -
Granted	20,000	25.06
Exercised	-	
Forfeited	-	
Unvested as of December 31, 2020	20,000	25.06
Granted	3,500	62.32
Exercised	-	
Forfeited	-	
Unvested as of December 31, 2021	23,500	30.61

The intrinsic value of RSU's vested during the years ended December 31, 2021 and 2020 was $0.4 million and $0.5 million.

As of December 31, 2021, there was approximately $0.5 million of unrecognized compensation cost related to RSU's granted. This amount is expected to be recognized over a weighted average period of 2.8 years.

Warrants

A summary of the warrants outstanding at December 31, 2021 is as follows:

Price	Outstanding	Grant Date	Expiration Date
24.42	7,051	June 28, 2013	June 28, 2023
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
Total	1,218,038		

The following table below is the summary of changes in warrants to purchase common stock for the years ended December 31, 2021 and 2020:

	Number of Warrant Shares Outstanding and Exercisable	Exercise Price per Share
Balance as of December 31, 2019	14,102	$ 24.42
Granted	603,386	$ 57.11
Exercised	(7,051)	$ -
Balance as of December 31, 2020	610,437	
Granted August 2021	301,291	$ 33.63
Granted December 2021	306,310	$ 22.51
Exercised	-	$ -
Balance as of December 31, 2021	1,218,038	

The Company issued warrants to a related party during the years ended December 31, 2021 and 2020 and incurred a non-cash R&D expense of $12.8 million and $26.6 million respectively (see Note 15- Related Party Transactions). During the year ended December 31, 2020, 7,051 of warrants were exercised into 1,601 shares of common stock.

The fair value of the 2021 and 2020 warrants was calculated using the Black-Scholes fair value pricing model with the following inputs:

	Years Ended December 31,			
	2021		2020	
Risk-free interest rate	1.37	%	0.95	%
Expected term (in years)	10.00		10.00	
Expected volatility	70.25	%	74.20	%
Expected dividend yield	0.00	%	0.00	%
Exercise price	$ 28.07		$ 57.11	
Fair value of common stock	$ 28.07		$ 57.11	

10. 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,		
	2021	2020	2019
Numerator:			
Net loss attributable to common stockholders	$ (169,069)	$ (139,700)	$ (77,270)
Denominator:			
Weighted-average common shares outstanding - basic and diluted	63,235,417	55,380,740	49,010,358
Net loss per share attributable to common stockholders - basic and diluted	$ (2.67)	$ (2.52)	$ (1.58)

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,		
	2021	2020	2019
Shares issuable upon conversion of the 2021 Convertible Notes	-	160,536	1,620,948
Shares issuable upon conversion of the 2022 Convertible Notes	-	1,195,449	-
Warrants exercisable for common shares	1,218,038	610,437	14,102
Restricted stock units exercisable for common shares	23,500	-	-
Options to purchase common shares	11,143,761	11,050,931	9,608,537
	12,385,299	13,017,353	11,243,587

11. Income Taxes

No provision for federal or state income taxes was recorded during the years ended December 31, 2021, 2020 and 2019, 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,		
	2021	2020	2019
U.S. federal tax at statutory rate	21.0%	21.0%	21.0%
Foreign rate differential	(13.0)%	0.0%	0.0%
Change in state tax apportionment	0.1%	0.1%	(17.4)%
Stock compensation	1.5%	0.9%	(0.7)%
Transfer pricing adjustments	(22.8)%	0.0%	0.0%
Valuation allowance	4.6%	(35.0)%	(5.1)%
Tax credits	8.7%	13.5%	0.0%
Other	(0.2)%	(0.5)%	2.1%
Effective tax rate	0%	0%	0%

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

	At December 31,		
	2021	2020	2019
Deferred income tax assets (liabilities)			
R&D Credits	$ 35,766	$ 42,613	$ 18,471
Net operating losses ("NOL") and credit carryforwards	26,789	21,359	2,517
Capitalized research and development costs	19,753	23,179	27,652
Stock-based compensation	11,552	7,406	3,896
Debt discount	-	(748)	(1,460)
Warrants	8,382	5,585	-
Other	(8,881)	1,189	643
Valuation allowance	(93,361)	(100,583)	(51,719)
Net deferred income tax asset	$ -	$ -	$ -

As of December 31, 2021, the Company had federal and state net operating loss carryforwards of approximately $118.2 million and $30.9 million, respectively, which begin to expire in 2026. Additionally, $85.7 million of the Federal NOLs can be carried forward indefinitely. The Company has federal R&D credits of $35.8 million which will begin to expire in 2038.

As required by ASC 740, *Income Taxes*, management of Rocket 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 Rocket has incurred tax losses from inception, management has determined that it is more likely than not that Rocket 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, 2021, 2020 and 2019. Rocket 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, 2021 and 2020, the valuation allowance decreased by $7.2 million and increased by $48.9 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.

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 our 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 Rocket 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 our 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, 2021, 2020 and 2019, 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 statement of operations. As of December 31, 2021, 2020 and 2019, no accrued interest or penalties are included on the related tax liability line in the consolidated balance sheets. The Company was audited by New York City for the 2017 tax year and the audit was completed with no change to the 2017 New York City tax return.

12. Commitments and Contingencies

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.

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. A smaller area within this facility was originally leased in August 2018, and the lease was amended in June 2019 to include the full building (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.

The Company determined the lease commencement date was reached on March 15, 2020, when the construction of all landlord owned improvements had been substantially completed and when the Company began including its leasehold improvements on the balance sheet and move equipment into the space. Upon commencement of the NJ Lease Agreement, the Company recognized total right-of-use assets of $47.7 million, with a corresponding lease liability of $20.2 million. The Company reclassified $26.5 million of construction costs in progress and $1.1 million of prepaid rent as part of the right-of-use asset upon the lease commencement date of March 15, 2020. During the year ended December 31, 2021, the Company reclassified an additional $0.1 million of construction costs in progress bringing the aggregate reclassification through December 31, 2021, of $32.1 million of construction costs in progress as part of the right of use asset.

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 in deposits in the consolidated balance sheets as of December 31, 2021 and 2020.

The total restricted cash balance for the Company's operating and finance leases at December 31, 2021 and 2020 was $0.8 million and $1.1 million, respectively.

Operating Leases

On March 31, 2016, the Company entered into a lease agreement for its office and laboratory space at the Alexandria Center for Life Sciences in New York, New York with an initial term ending on July 31, 2022 (the "NY Lease Agreement"). Effective May 31, 2020, the Company terminated the NY Lease Agreement and recorded a loss on lease termination of $76 for the year ended December 31, 2020.

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 in 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 accounted for the ESB Lease Amendment as a modification to the ESB Lease Agreement and remeasured the lease liability and adjusted the operating lease right of use asset by $1.1 million.

The Company has a certificate of deposit of $0.8 million and $0.9 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, 2021 and December 31, 2020, respectively.

On January 4, 2018, in connection with the Reverse Merger, the Company assumed an operating lease for Inotek's former headquarters in Lexington, Massachusetts, with a term ending in February 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 $0.4 million, $0.4 million, and $0.3 million for the years ended December 31, 2021, 2020 and 2019, respectively. These amounts are netted against rent expense in the consolidated statement of operations for the years ended December 31, 2021, 2020 and 2019.

Rent expense was $1.2 million, $1.1 million, and $0.9 million for the years ended December 31, 2021, 2020 and 2019, respectively.

Lease cost	**December 31, 2021**
Operating lease cost	$ 645
Finance lease cost	
Amortization of right of use assets	2,140
Interest on lease liablities	1,845
Total lease cost	$ 4,630

The following table summarizes the maturity of the Company's lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating lease liabilities recognized on the Company's balance sheet as of December 31, 2021:

Maturity of operating lease liabilities	**December 31, 2021**
2022	917
2023	548
2024	269
2025	64
2026	54
Total lease payments	$ 1,852
Less: interest	(84)
Total operating lease liabilities	$ 1,768

Maturity of finance lease liability	**December 31, 2021**
2022	1,689
2023	1,736
2024	1,791
2025	1,856
2026	1,912
Thereafter	45,000
Total lease payments	$ 53,984
Less: interest	(33,151)
Total finance lease liability	$ 20,833

Leases	**December 31, 2021**
Operating right-of-use assets	$ 1,569
Operating current lease liabilities	863
Operating noncurrent lease liabilities	905
Total operating lease liabilities	$ 1,768
Finance right-of-use assets	$ 48,480
Finance current lease liability	1,689
Finance noncurrent lease liability	19,144
Total finance lease liability	$ 20,833

Other information

Cash paid for amounts included in the measurement of lease liabilities:

Operating cash flows from operating leases	$	655
Cash flows from finance lease	$	1,644
Weighted-average remaining lease term - operating leases		2.5 years
Weighted-average remaining lease term - finance lease		22.7 years
Weighted-average discount rate - operating leases		4.46%
Weighted-average discount rate - finance lease		8.96%

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.

13. 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 Agreement for Danon Disease 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 Danon disease. 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. 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 Danon disease. 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 Danon disease 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 expires 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 included as research and development expenses. A fee of $2.0 million per additional vector would be due if the Company exercises its 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 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 European Union. 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 a $1.0 million license fee payment under the RGNX agreement upon the dosing of the first Danon patient in 2019. There were no additional milestones achieved or related payments made during the years ended December 31, 2021 and 2020.

14. CIRM Grants

LAD-I CIRM Grant

On April 30, 2019, the California Institute for Regenerative Medicine ("CIRM") awarded the Company up to $6.5 million under a CLIN2 grant award to support the clinical development of its LVV-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, University of California, Los Angeles ("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. In 2019, the Company received the first two grants from CIRM in the aggregate of $1.2 million which were included as an offset against R&D expenses. In 2020, the Company met additional CIRM milestones and received an additional $1.1 million milestone which was recorded as a reduction of R&D expenses in 2020. The Company received the additional milestone payments of $1.1 million and $1.0 million in January and April of 2021, respectively. As of December 31, 2021, the Company did not meet the next milestone and therefore no receivable has been recorded.

IMO CIRM Grant

On November 12, 2020, the CIRM awarded the Company up to $3.7 million under a CLIN2 grant award to support the clinical development of its LVV-based gene therapy, RP-L401, for the treatment of IMO. The Company received $1.0 million pursuant to the grant on January 4, 2021, related to the CIRM IMO award and recorded a receivable, included in prepaid and other assets in the consolidated balance sheet, and a reduction of research and development expenses of $0.9 million as of December 31, 2020. The Company recorded a reduction of research and development expense of $0.1 million for the year ended December 31, 2021. As of December 31, 2021, the Company did not meet the next milestone and no receivable has been recorded. Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program is to be returned to academic innovators.

15. Related Party Transactions

During April 2018, the Company entered into an agreement with a member of the Board of Directors for business development consulting services. Payments for the services under the agreement are $28 per quarter, and the Company may terminate the agreement with 14 days' notice. The Company incurred expenses of $0.1 million for each of the years ended December 31, 2021, 2020 and 2019, relating to services provided under this agreement.

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 October 2020, the Company entered into a consulting agreement with the spouse of one of the Company's executive officers for information technology advisory services. In exchange for the services provided under the agreement, the Company granted 10,000 restricted stock units which vest over a three-year period.

On August 27, 2021, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with a fund affiliated with RTW Investments, LP, the Company's largest shareholder (the "Purchaser"), pursuant to which it agreed to sell and issue to the Purchaser, in a private placement (the "Private Placement"), 812,516 shares of the Company's common stock at a purchase price of $32.48 per share for aggregate net proceeds of approximately $26.4 million to the Company before deducting estimated offering expenses payable by the Company. The Private Placement closed on August 31, 2021. In addition, concurrently with the execution of the Purchase Agreement, the Company entered into a registration rights agreement with the Purchaser, pursuant to which the Company agreed, following demand by the Purchaser, to file with the Securities and Exchange Commission a Registration Statement on Form S-3 covering the resale of shares of common stock held by the Purchaser as promptly as reasonably practicable following such demand, and in any event within 60 days of such demand.

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. The term of the consulting agreement is three years and may be terminated with 60 days' notice by either party. In exchange for the business development services to be provided under the agreement, the Company issued a warrant exercisable for 603,386 shares of Common Stock. Pursuant to the consulting agreement, the related party is entitled to receive additional warrants exercisable for common stock upon identification of new assets for the Company to in-license. The Company recorded a non-cash R&D expense of $26.6 million for the year ended December 31, 2020, related to the issuance of the Common Stock warrant.

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.

16. 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 to match 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the years ended December 31, 2021, 2020, and 2019 was $0.6 million, $0.3 million, and $0.2 million, respectively.

Exhibit 10.10

Rocket Pharmaceuticals LTD
c/o CO Services Cayman Limited
P.O. Box 10008
Willow House
Cricket Square
Grand Cayman KYI-1001
Cayman Islands

September 29, 2017

Dear Raj Prabhakar:

Rocket Pharmaceuticals LTD (the "Company") and the Company's Board of Directors (the "Board") are pleased to offer you the following tem1s of employment as Senior Vice-President, Business Operations and Corporate Strategy for the Company, effective date Monday October 23, 2017 (or alternative if prior agreement reached with manager), reporting to the CEO and Board of Directors. The initial terms of your new position with the Company are as set forth below.

1. Position.

You will be the Senior Vice-President, Business Operations and Corporate Strategy for the Company. Your responsibilities in this position will include but not be limited to leading negotiations, contracting, and operational execution for duties such as strategic transactions; vendor relationships; material transfer agreements; in-licensing; out-licensing; collaboration; and consulting arrangements. Additional responsibilities included financial oversight for contracts and partnerships; lead intellectual property strategy; collaborate with partners and R&D teams to assist in project management; assist in maintaining relationships with key opinion leaders; assist with company finances, valuation and M&A; and assist in communication with investors. You agree to the best of your ability and experience that you will at all times loyally and conscientiously perform all of the duties and obligations required of and from the Company. During the term of your employment, you further agree that you will devote all of your business time and attention to the business of the Company and that you will not, directly or indirectly, engage or participate in any personal, business, charitable or other enterprise that is competitive in any manner with the business of the Company, whether or not such activity is for compensation. As part of your personal and career development, subject to CEO and Board of Director approval, you will be permitted to serve either in an Executive or Board Director capacity on industry, academic or non-profit entities such as ARM (Alliance for Regenerative Medicine) or other similar non-profit groups that may be beneficial to performing duties *in* the cell and gene therapy field, as well as alumni or related non-profit groups for which you have personal interest.

2. Compensation.

Your base salary will be at the rate of $270,000 per year, less payroll deductions and all required withholdings. You will be paid semi-monthly on the Company's regularly scheduled pay dates. In addition, each calendar year, you will be eligible to earn an additional cash bonus with a target bonus of 38% of your base salary, based on the Company's assessment of your individual performance and overall Company performance. In order to earn and receive the bonus, you must remain employed by the Company through and including the bonus payout date, which will be on or before March 15 of the year following the year for which it is paid. The determination of whether you have earned a bonus and the amount thereof shall be determined by the Board (and/or a committee thereof) in its sole and absolute discretion. Your bonus for 2017 shall be on a pro rata basis based on your start date (as defined below).

You will also receive a sign on bonus of $15,000 to be paid on the company's next regularly scheduled pay date following your start date. Prior to one year following your start date, in the event you elect to terminate your employment with the Company without Good Reason or your employment is terminated by the Company with Cause, then you are responsible for reimbursement of the sign on bonus to the Company within 90 days following the date of termination.

You will receive three (3) weeks of vacation annually. Your vacation will be prorated for 2017 based on your start date. Beyond 2017, your vacation will vest according to company policy with starting base of 3 weeks being in effect and up to one week of vacation carry over to the next year.

In addition, you will, in accordance with Company policy and the terms of the applicable plan documents, be eligible to participate in benefits under any benefit plan or arrangement that may be in effect from time to time and made available to similarly situated Company employees. The Company reserves the right to modify, add or eliminate benefits from time to time. Your healthcare coverage benefits will commence on the first day of your employment.

You will also be eligible to participate in a Company-sponsored 40l(k) plan with 4% Company match and immediate 100% vesting.

3. Stock Options Grant.

You will be issued Stock Options to purchase 2,170 of the Company's Ordinary Shares ("Options") pursuant to the Company's 2015 Share Option Plan The Company's Options shall vest as follows: 1/3 shall vest upon the one-year anniversary of your Start Date (defined below) and one-eighth (1/8) of the remaining 2/3 Options shall vest at the end of each quarter beginning with the end of the first full calendar quarter following the one-year anniversary of your Start Date. The Options shall be subject to the applicable terms of the Options Agreement, including applicable expiration period. Subject to the discretion of the Board of Directors of the Company, and based on multiple factors including, but not limited to, individual performance and dilution consideration, you will be eligible for additional grants of options pursuant to the 2015 Option Plan.

4. At-Will Employment & Termination.

Your employment will commence October 23, 2017 (or alternative if prior agreement reached with manager) (the "Start Date"). Your employment with the Company will be "at-will." This means that either you or the Company may terminate your employment relationship at any time, with or without notice, and with or without Cause. Provided you sign and allow to become effective, a general release of claims in favor of the Company within the timeframe set forth in the form of release to be provided by the Company at or around the time of employment termination, then:

- In the event your employment is terminated by the Company without Cause on or after the first-year anniversary of your Start Date then in addition to the Options that shall have vested as of the date of termination, the Options that would have vested on the last day of the calendar quarter in which such termination occurs shall vest.

- In the event your employment is terminated by the Company without Cause or you terminate your employment for Good Reason on or after the first-year anniversary of your Start Date, then the Company shall pay you your base salary for the six months following such termination.

If your employment is terminated by the Company for Cause, your Options, whether vested or unvested, shall terminate and not be exercisable and you will not be eligible to receive the six months base salary payment following such termination noted above.

For purposes of this paragraph 4, "Cause" for the Company to terminate your employment shall mean the occurrence of any of the following subsequent to starting employment at the Company:

(i)your conviction of any felony or any crime involving fraud or dishonesty; (ii) your participation in a fraud, act of dishonesty or other act of gross misconduct against the Company; (iii) your violation of any statutory or fiduciary duty, or duty of loyalty, owed to the Company; (iv) your breach of any material term of any contract between such you and the Company; or (v) your material violation of Company policy. Whether a termination is for Cause shall be decided by the Board in its sole and exclusive judgment and discretion.

For purposes of this paragraph 4, "Good Reason'' shall mean the occurrence of any of the following: (i) the Company's breach of any material term of this letter or (ii) a material reduction in your duties or authority as described in paragraph 1 above.

5. Additional Information.

This letter contains all of the terms of your employment with the Company and supersede any prior understandings or agreements, whether oral or written, between you and the Company. This letter may not be amended or modified except by an express written agreement signed by you and the Board. This offer is contingent upon: (i) your providing proof of your right to work in the United States; (ii) your signing the enclosed Proprietary Information; Inventions Assignment Agreement; and Non-Solicitation/Non-Competition agreement; and (iii) if requested by the Company, your authorizing the Company to conduct a background check and satisfactory results of such check.

Sincerely,

Gaurav Shah, M.D.

Chief Executive Officer of Rocket Pharmaceuticals LTD.

Agreed and accepted:

Raj Prabhakar

Exhibit 10.12

350 Fifth Avenue, Suite 7530
New York, NY 10118

9 Cedar Brook Drive
Cranbury, NJ 08512

Tel: (646) 440-9100
Fax: (646) 224-9585

April 29, 2021

Isabel Carmona

Dear Isabel,

Rocket Pharmaceuticals, Inc. is pleased to offer you a position as SVP, Chief Human Resources Officer starting on June 28, 2021, reporting to Gaurav Shah, CEO. This is a full-time position located at our Cranbury, NJ location.

The starting base salary will be $350,000 per year, and you will be eligible for an annual discretionary bonus of up to 35% of your full year base salary payable in 2022 for your 2021 performance.

You will also have healthcare benefits from the first day of employment, the opportunity to participate in a Rocket-sponsored 401(k) plan with 4% match and immediate 100% vesting, and 17 days of paid vacation annually. Our schedule of paid holidays includes a Rocket-wide winter break from December 25 – January 1 of each year.

In addition, subject to applicable approval by the Board of Directors, you will be issued stock options to purchase 75,000 common ordinary shares of Rocket Pharmaceuticals, Inc. pursuant to the applicable share option plan and your option agreement. Employee shares vest over a three year period: 1/3 after one year, and the remaining 2/3 vesting quarterly over the subsequent two years.

Your employment will be “at-will.” This means that either you or Rocket may terminate your employment at any time, with or without notice, and with or without cause. This letter does not constitute a binding agreement and is not an employment contract, and Rocket may change your terms and conditions of employment at any time.

Your employment at Rocket is contingent upon: (i) your providing proof of your right to work in the United States; (ii) your signing our standard Proprietary Information; Inventions Assignment Agreement; and Non-Solicitation/Non-Competition agreement; and (iii) if requested by Rocket, satisfactory results of a drug test and/or background test.

Please confirm your acceptance of this offer by signing and returning this letter by April 30, 2021.

We look forward to welcoming you to Rocket!

Sincerely,

Gaurav D. Shah, MD

President & CEO,
Rocket Pharmaceuticals, Inc.

Agreed and accepted:

_____ Date: _____

[New Employee]

Exhibit 10.26

THIS WARRANT AND THE SHARES ISSUABLE UPON THE EXERCISE HEREOF HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED, HYPOTHECATED, OR OTHERWISE TRANSFERRED EXCEPT PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933, AS AMENDED OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY THAT REGISTRATION IS NOT REQUIRED.

ROCKET PHARMACEUTICALS, INC.

WARRANT TO PURCHASE SHARES

This Warrant (this "Warrant") is issued to Neptune Consulting, LLC ("Neptune") by Rocket Pharmaceuticals, Inc., a Delaware corporation (the "Company"), as of December 17, 2021 (the "Effective Date"), in connection with the holder's provision of consulting services to the Company pursuant to the consulting agreement, dated as of December 18, 2020 (the "Consulting Agreement").

1. Purchase of Shares. Subject to the terms and conditions hereinafter set forth, the holder of this Warrant is entitled, upon surrender of this Warrant at the principal office of the Company (or at such other place as the Company shall notify the holder hereof in writing), to purchase from the Company up to 153,155 shares, as adjusted pursuant to Section 7 below (the "Shares"), of the Company's common stock, par value $0.01 per share (the "Common Stock"), at the Exercise Price (as defined below). Any future warrants granted pursuant to the Consulting Agreement may provide for more or less shares.

2. Definitions.

   a. Change of Control. The term "Change of Control" shall mean (i) the acquisition of the Company by another entity by means of any transaction or series of related transactions with the Company (including, without limitation, any stock purchase, reorganization, merger or consolidation but, excluding any merger effected exclusively for the purpose of changing the domicile of the Company); or (ii) a sale of all or substantially all of the assets of the Company, unless the Company's stockholders of record as constituted immediately prior to such acquisition or sale will, immediately after such acquisition or sale (solely by virtue of securities issued as consideration for the Company's acquisition or sale or otherwise) hold at least 50% of the voting power of the surviving or acquiring entity.

   b. Exercise Price. The exercise price for the Shares shall be $22.51 per Share, as adjusted for any stock splits, dividends, combinations and the like as provided in Section 7 below (such price, as adjusted from time to time, is herein referred to as the "Exercise Price").

3. Change of Control. In the event of a Change of Control, the holder of this Warrant shall have the right thereafter to receive, upon exercise of this Warrant, the same amount and kind of securities, cash or property as it would have been entitled to receive upon the occurrence of such Change of Control if it had been, immediately prior to such Change of Control, the holder of the number of Warrant Shares then issuable upon exercise in full of this Warrant without regard to any limitations on exercise contained herein, and the holder of this Warrant shall no longer have the right to receive Warrant Shares upon exercise of this Warrant.

4. Exercise.

    a. Method of Exercise. While this Warrant remains outstanding and exercisable, the holder may exercise, in whole or in part, the purchase rights evidenced hereby. Such exercise shall be effected by:

        i. the surrender of the Warrant, together with a notice of exercise to the Secretary of the Company at its principal offices; and

        ii. the payment to the Company of an amount equal to the aggregate Exercise Price for the number of Shares being purchased.

        iii. In lieu of exercising this Warrant as specified in this Section 4(a)(ii), the holder may convert this Warrant, in whole or in part, into a number of Shares determined by dividing (a) the aggregate fair market value of the Shares issuable upon exercise of this Warrant (or lesser number of shares in the case of a partial exercise) minus the aggregate Exercise Price of such Shares by (b) the fair market value of one Share. The fair market value of the Shares shall be the closing price of the Shares reported for the business day immediately before the holder delivers its notice of exercise to the Company

    b. Exercise Period. This Warrant shall be immediately exercisable upon the Effective Date in the amount of 153,155 Shares. This Warrant shall cease to be exercisable upon the expiration of this Warrant pursuant to Section 16 hereof.

5. Certificates for Shares. Upon the exercise of the purchase rights evidenced by this Warrant, one or more certificates or book-entry notations for the number of Shares so purchased and, if this Warrant has not been fully exercised or converted and has not expired, a new Warrant representing the Shares not so acquired, shall be issued as soon as practicable thereafter.

6. Issuance of Shares. The Company covenants that the Shares, when issued pursuant to the exercise of this Warrant, will be duly and validly issued, fully paid and nonassessable.

7. Adjustment of Exercise Price and Number of Shares. The number of and kind of securities purchasable upon exercise of this Warrant and the Exercise Price shall be subject to adjustment from time to time as follows:

a. Subdivisions, Combinations and Other Issuances. If the Company declares or pays a dividend or distribution on the shares of Common Stock payable in common stock or other securities or property (other than cash), then upon exercise of this Warrant, for each Share acquired, the holder shall receive, without additional cost to the holder, the total number and kind of securities and property which holder would have received had the holder owned the Shares of record as of the date the dividend or distribution occurred. If the Company subdivides the outstanding shares of Common Stock by reclassification or otherwise into a greater number of shares, the number of Shares purchasable hereunder shall be proportionately increased and the Exercise Price shall be proportionately decreased. If the outstanding shares of Common Stock are combined or consolidated, by reclassification or otherwise, into a lesser number of shares, the Exercise Price shall be proportionately increased and the number of Shares shall be proportionately decreased. Any adjustment under this Section 7(a) shall become effective at the close of business on the date the subdivision or combination becomes effective, or as of the record date of such dividend, or in the event that no record date is fixed, upon the making of such dividend.

b. Reclassification, Reorganization and Consolidation. Upon any event whereby all of the outstanding shares of Common Stock are reclassified, exchanged, combined, substituted, or replaced for, into, with or by Company securities of a different class and/or series, then from and after the consummation of such event, this Warrant will be exercisable for the number, class and series of Company securities that holder would have received had the Shares been outstanding on and as of the consummation of such event, and subject to further adjustment thereafter from time to time in accordance with the provisions of this Warrant.

c. Notice of Adjustment and Certain Events. The Company shall provide the holder with not less than 5 days prior written notice of, including a description of the material facts surrounding, any of the following events: (a) declaration of any dividend or distribution upon its Common Stock, whether in cash, property, stock, or other securities and whether or not a regular cash dividend; (b) offering for subscription pro rata to the holders of any class or series of its stock any additional shares of stock of any class or series or other rights; (c) effecting any reclassification or recapitalization of Common Stock; (d) the merger or consolidation with or into any other corporation, or sale, lease, license, or conveyance of all or substantially all of its assets, or liquidation, dissolution or winding up; or (e) when any other adjustment is required to be made in the number or kind of shares purchasable upon exercise of this Warrant, or in the Exercise Price. When any adjustment is required to be made in the number or kind of shares purchaseable upon exercise of this Warrant, or in the Exercise Price, the Company shall promptly compute such adjustment or readjustment in accordance with the provisions hereof and prepare a certificate showing such adjustment or readjustment, and shall mail such certificate to the holder of this Warrant in accordance with the notice provisions of Section 17. The certificate shall set forth such adjustment or readjustment and indicate the number of shares of Common Stock and the Exercise Price in effect after such adjustment or readjustment. The provisions of this Section 7(c) shall similarly apply to successive reclassifications, exchanges, combinations substitutions, replacements or other similar events.

8. No Fractional Shares or Scrip. No fractional shares or scrip representing fractional shares shall be issued upon the exercise of this Warrant, but in lieu of such fractional shares the Company shall make a cash payment therefor on the basis of the Exercise Price then in effect.

9. Reservation of Shares. During the period between the Effective Date and the Expiration Date, the Company shall at all times reserve and keep available out of its authorized but unissued shares of Common Stock or other securities constituting Shares, solely for the purpose of issuance upon the exercise of this Warrant, the maximum number of Shares issuable upon the exercise of this Warrant, and the par value per Share shall at all times be less than or equal to the applicable Exercise Price.

10. Representations of the Company. The Company represents that all corporate actions on the part of the Company, its officers, directors and stockholders necessary for the sale and issuance of this Warrant have been taken. The Company will pay all original issue and transfer taxes, if any, with respect to the issue and delivery of the Shares pursuant hereto and all other fees and expenses necessarily incurred by the Company in connection herewith.

11. Representations and Warranties by the Holder. The holder of this Warrant represents and warrants to the Company as follows:

    a. This Warrant and the Shares issuable upon exercise thereof are being acquired for its own account, for investment and not with a view to, or for resale in connection with, any distribution or public offering thereof within the meaning of the Securities Act of 1933, as amended (the "Act"). Upon exercise of this Warrant, the holder of this Warrant shall, if so requested by the Company, confirm in writing, in a form satisfactory to the Company, that the securities issuable upon exercise of this Warrant are being acquired for investment and not with a view toward distribution or resale.

    b. The holder of this Warrant understands that this Warrant and the Shares have not been registered under the Act by reason of their issuance in a transaction exempt from the registration and prospectus delivery requirements of the Act pursuant to Section 4(a)(2) thereof, and that they must be held by the holder indefinitely, and that the holder must therefore bear the economic risk of such investment indefinitely, unless a subsequent disposition thereof is registered under the Act or is exempted from such registration.

c. The holder of this Warrant has such knowledge and experience in financial and business matters that it is capable of evaluating the merits and risks of the purchase of this Warrant and the Shares purchasable pursuant to the terms of this Warrant and of protecting its interests in connection therewith.

d. The holder of this Warrant is aware of the Company's business affairs and financial condition and has received or has had full access to all the information it considers necessary or appropriate to make an informed investment decision with respect to the acquisition of this Warrant and its underlying securities. The holder of this Warrant further has had an opportunity to ask questions and receive answers from the Company regarding the terms and conditions of the offering of this Warrant and its underlying securities and to obtain additional information (to the extent the Company possessed such information or could acquire it without unreasonable effort or expense) necessary to verify any information furnished to the holder of this Warrant or to which the holder has access.

e. The holder of this Warrant is able to bear the economic risk of the purchase of the Shares pursuant to the terms of this Warrant.

f. The holder of this Warrant is an "accredited investor" as such term is defined in Rule 501 of Regulation D promulgated under the Act.

12. Restrictive Legend. The Shares shall be stamped or imprinted with a legend in substantially the following form:

THE SHARES REPRESENTED BY THIS CERTIFICATE HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF, AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED. SUCH SHARES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR AN EXEMPTION THEREFROM UNDER THE ACT AND ANY APPLICABLE STATE SECURITIES LAWS. COPIES OF THE AGREEMENT COVERING THE PURCHASE OF THESE SHARES AND RESTRICTING THEIR TRANSFER MAY BE OBTAINED AT NO COST BY WRITTEN REQUEST MADE BY THE HOLDER OF RECORD OF THIS CERTIFICATE TO THE SECRETARY OF THE COMPANY AT THE PRINCIPAL EXECUTIVE OFFICES OF THE COMPANY.

13. Warrants Transferable.

a. This Warrant, and any Shares issued hereunder, may not be sold, assigned or otherwise transferred by the holder hereof until six months after the Effective Date, except for an assignment or transfer of this Warrant, in whole or in part, by Neptune to an affiliate of Neptune, excluding any affiliate of the Company that is a natural person. Thereafter, this Warrant and the Shares may be offered, sold or otherwise transferred or disposed, in whole or in part, by the holder. Any sale, transfer or assignment of this Warrant or the Shares issued hereunder shall be subject to compliance with the terms and conditions of Section 13(b) and applicable federal and state securities laws.

b. Any transfer of this Warrant shall require surrender of this Warrant properly endorsed or accompanied by written instructions of transfer. With respect to any offer, sale or other disposition of this Warrant or the Shares, the holder hereof agrees to give written notice to the Company prior thereto, describing briefly the manner thereof, together with a written opinion of the holder's counsel, or other evidence, if requested by the Company, to the effect that such offer, sale or other disposition may be effected without registration or qualification (under the Act as then in effect or any federal or state securities law then in effect) of this Warrant or the Shares and indicating whether or not under the Act certificates for this Warrant or the Shares to be sold or otherwise disposed of require any restrictive legend as to applicable restrictions on transferability in order to ensure compliance with such law; provided, however, that (i) the Company shall not require the holder to provide an opinion of counsel if the transfer is to any affiliate of the holder, provided that any such transferee is an "accredited investor" as defined in Regulation D promulgated under the Act, and additionally, (ii) the Company shall also not require an opinion of counsel if there is no material question as to the availability of Rule 144 promulgated under the Act. Upon receiving such written notice and reasonably satisfactory opinion or other evidence, if so requested, the Company, as promptly as practicable, shall notify the holder that the holder may sell or otherwise dispose of this Warrant or the Shares, all in accordance with the terms of the notice delivered to the Company. If a determination has been made pursuant to this Section 13 that the opinion of counsel for the holder or other evidence is not reasonably satisfactory to the Company, the Company shall so notify the holder promptly with details thereof after such determination has been made. Each certificate representing this Warrant or Shares transferred in accordance with this Section 13 shall bear a legend as to the applicable restrictions on transferability in order to ensure compliance with such laws, unless in the aforesaid opinion of counsel for the holder, such legend is not required in order to ensure compliance with such laws. The Company may issue stop transfer instructions to its transfer agent in connection with such restrictions.

14. Reserved.

15. No Rights as Stockholder. The holder of this Warrant, as a holder of this Warrant, will not have any voting rights or other rights of a stockholder of the Company until exercise of this Warrant.

16. Expiration of Warrant. This Warrant shall be exercisable in whole or in part, at any time and from time to time, until ten years from the original issue date of this Warrant (the "Expiration Date"). If this Warrant has not been exercised prior to the Expiration Date, this Warrant shall be deemed to have been automatically exercised on the Expiration Date by "cashless" conversion pursuant to Section 4(a)(iii).

17. Notices. Any notice, consent, claim, demand, waiver, or other communication under this Warrant have legal effect only if in writing and addressed to a party to the address set forth below (or to such other address or such other person that a party may designate for itself from time to time in accordance with this Section). Notices sent in accordance with this Section will be deemed effectively given: (a) when received, if delivered by hand, with signed confirmation of receipt; (b) when received, if sent by a nationally recognized overnight courier, signature required; (c) when sent, if by email, (in each case, with confirmation of transmission), if sent during the addressee's normal business hours, and on the next business day, if sent after the addressee's normal business hours; and (d) on the third day after the date mailed by certified or registered mail, return receipt requested, postage prepaid.

Neptune:
c/o RTW Investments, LP
40 10th Avenue, Floor 7
New York, NY 10014
Attention: Legal Department

with a copy to Neptune's counsel:
Gibson, Dunn & Crutcher LLP
555 Mission Street
Suite 3000
San Francisco, CA 94105
Attention: Ryan A. Murr

Company:
Rocket Pharmaceuticals, Inc.
9 Cedar Brook Drive
Cranbury, NJ 08512
Attention: Chief Executive Officer

18. Governing Law. This Warrant and all actions arising out of or in connection with this Agreement shall be governed by and construed in accordance with the laws of the State of New York, without regard to the conflicts of law provisions of the State of New York or of any other state.

19. Rights and Obligations Survive Exercise of Warrant. Unless otherwise provided herein, the rights and obligations of the Company, of the holder of this Warrant and of the holder of the Shares issued upon exercise of this Warrant, shall survive the exercise of this Warrant.

[*Signature page follows*]

IN WITNESS WHEREOF, the Company has caused this Warrant to be executed by its officer thereunto duly authorized as of the date first above indicated.

ROCKET PHARMACEUTICALS, INC.

By: _____

Name: Gaurav Shah
Title: President & CEO

EXHIBIT A

NOTICE OF EXERCISE

TO: ROCKET PHARMACEUTICALS, INC.

Attention: Chief Executive Officer

1. The undersigned hereby elects to purchase_____Shares pursuant to the terms of the attached Warrant.

2. Method of Exercise (Please initial the applicable blank):

☐ The undersigned elects to exercise the attached Warrant by means of a cash payment, and tenders herewith payment in full for the purchase price of the shares being purchased, together with all applicable transfer taxes, if any.

☐ The undersigned elects to exercise the attached Warrant by means of the net exercise provisions of Section 4(a)(iii) of the Warrant.

3. Please issue a certificate or certificates representing said Shares in the name of the undersigned or in such other name as is specified below:

_____

(Name)

_____

(Address)

4. The undersigned hereby represents and warrants that all representations and warranties of the undersigned set forth in Section 11 of the attached Warrant (including Section 11(f) thereof) are true and correct as of the date hereof.

_____

(Signature)

_____

(Name)

_____

(Title)

_____

(Date)

EXHIBIT B

FORM OF TRANSFER
(To be signed only upon transfer of Warrant)

FOR VALUE RECEIVED, the undersigned hereby sells, assigns and transfers unto_____the right represented by the attached Warrant to purchase_____shares of Common Stock of Rocket Pharmaceuticals, Inc. to which the attached Warrant relates, and appoints_____Attorney to transfer such right on the books of_____, with full power of substitution in the premises.

Dated:_____

(Signature must conform in all respects to name of Holder as specified on the face of the Warrant)
Address:_____

Signed in the presence of:

_____

_____

Exhibit 10.27

THIS WARRANT AND THE SHARES ISSUABLE UPON THE EXERCISE HEREOF HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED, HYPOTHECATED, OR OTHERWISE TRANSFERRED EXCEPT PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933, AS AMENDED OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY THAT REGISTRATION IS NOT REQUIRED.

ROCKET PHARMACEUTICALS, INC.

WARRANT TO PURCHASE SHARES

This Warrant (this "Warrant") is issued to Neptune Consulting, LLC ("Neptune") by Rocket Pharmaceuticals, Inc., a Delaware corporation (the "Company"), as of December 17, 2021 (the "Effective Date"), in connection with the holder's provision of consulting services to the Company pursuant to the consulting agreement, dated as of December 18, 2020 (the "Consulting Agreement").

1. Purchase of Shares. Subject to the terms and conditions hereinafter set forth, the holder of this Warrant is entitled, upon surrender of this Warrant at the principal office of the Company (or at such other place as the Company shall notify the holder hereof in writing), to purchase from the Company up to 153,155 shares, as adjusted pursuant to Section 7 below (the "Shares"), of the Company's common stock, par value $0.01 per share (the "Common Stock"), at the Exercise Price (as defined below). Any future warrants granted pursuant to the Consulting Agreement may provide for more or less shares.

2. Definitions.

   a. Change of Control. The term "Change of Control" shall mean (i) the acquisition of the Company by another entity by means of any transaction or series of related transactions with the Company (including, without limitation, any stock purchase, reorganization, merger or consolidation but, excluding any merger effected exclusively for the purpose of changing the domicile of the Company); or (ii) a sale of all or substantially all of the assets of the Company, unless the Company's stockholders of record as constituted immediately prior to such acquisition or sale will, immediately after such acquisition or sale (solely by virtue of securities issued as consideration for the Company's acquisition or sale or otherwise) hold at least 50% of the voting power of the surviving or acquiring entity.

   b. Exercise Price. The exercise price for the Shares shall be $22.51 per Share, as adjusted for any stock splits, dividends, combinations and the like as provided in Section 7 below (such price, as adjusted from time to time, is herein referred to as the "Exercise Price").

3. Change of Control. In the event of a Change of Control, the holder of this Warrant shall have the right thereafter to receive, upon exercise of this Warrant, the same amount and kind of securities, cash or property as it would have been entitled to receive upon the occurrence of such Change of Control if it had been, immediately prior to such Change of Control, the holder of the number of Warrant Shares then issuable upon exercise in full of this Warrant without regard to any limitations on exercise contained herein, and the holder of this Warrant shall no longer have the right to receive Warrant Shares upon exercise of this Warrant.

4. Exercise.

    a. Method of Exercise. While this Warrant remains outstanding and exercisable, the holder may exercise, in whole or in part, the purchase rights evidenced hereby. Such exercise shall be effected by:

        i. the surrender of the Warrant, together with a notice of exercise to the Secretary of the Company at its principal offices; and

        ii. the payment to the Company of an amount equal to the aggregate Exercise Price for the number of Shares being purchased.

        iii. In lieu of exercising this Warrant as specified in this Section 4(a)(ii), the holder may convert this Warrant, in whole or in part, into a number of Shares determined by dividing (a) the aggregate fair market value of the Shares issuable upon exercise of this Warrant (or lesser number of shares in the case of a partial exercise) minus the aggregate Exercise Price of such Shares by (b) the fair market value of one Share. The fair market value of the Shares shall be the closing price of the Shares reported for the business day immediately before the holder delivers its notice of exercise to the Company

    b. Exercise Period. This Warrant shall be immediately exercisable upon the Effective Date in the amount of 153,155 Shares. This Warrant shall cease to be exercisable upon the expiration of this Warrant pursuant to Section 16 hereof.

5. Certificates for Shares. Upon the exercise of the purchase rights evidenced by this Warrant, one or more certificates or book-entry notations for the number of Shares so purchased and, if this Warrant has not been fully exercised or converted and has not expired, a new Warrant representing the Shares not so acquired, shall be issued as soon as practicable thereafter.

6. Issuance of Shares. The Company covenants that the Shares, when issued pursuant to the exercise of this Warrant, will be duly and validly issued, fully paid and nonassessable.

7. Adjustment of Exercise Price and Number of Shares. The number of and kind of securities purchasable upon exercise of this Warrant and the Exercise Price shall be subject to adjustment from time to time as follows:

a. Subdivisions, Combinations and Other Issuances. If the Company declares or pays a dividend or distribution on the shares of Common Stock payable in common stock or other securities or property (other than cash), then upon exercise of this Warrant, for each Share acquired, the holder shall receive, without additional cost to the holder, the total number and kind of securities and property which holder would have received had the holder owned the Shares of record as of the date the dividend or distribution occurred. If the Company subdivides the outstanding shares of Common Stock by reclassification or otherwise into a greater number of shares, the number of Shares purchasable hereunder shall be proportionately increased and the Exercise Price shall be proportionately decreased. If the outstanding shares of Common Stock are combined or consolidated, by reclassification or otherwise, into a lesser number of shares, the Exercise Price shall be proportionately increased and the number of Shares shall be proportionately decreased. Any adjustment under this Section 7(a) shall become effective at the close of business on the date the subdivision or combination becomes effective, or as of the record date of such dividend, or in the event that no record date is fixed, upon the making of such dividend.

b. Reclassification, Reorganization and Consolidation. Upon any event whereby all of the outstanding shares of Common Stock are reclassified, exchanged, combined, substituted, or replaced for, into, with or by Company securities of a different class and/or series, then from and after the consummation of such event, this Warrant will be exercisable for the number, class and series of Company securities that holder would have received had the Shares been outstanding on and as of the consummation of such event, and subject to further adjustment thereafter from time to time in accordance with the provisions of this Warrant.

c. Notice of Adjustment and Certain Events. The Company shall provide the holder with not less than 5 days prior written notice of, including a description of the material facts surrounding, any of the following events: (a) declaration of any dividend or distribution upon its Common Stock, whether in cash, property, stock, or other securities and whether or not a regular cash dividend; (b) offering for subscription pro rata to the holders of any class or series of its stock any additional shares of stock of any class or series or other rights; (c) effecting any reclassification or recapitalization of Common Stock; (d) the merger or consolidation with or into any other corporation, or sale, lease, license, or conveyance of all or substantially all of its assets, or liquidation, dissolution or winding up; or (e) when any other adjustment is required to be made in the number or kind of shares purchasable upon exercise of this Warrant, or in the Exercise Price. When any adjustment is required to be made in the number or kind of shares purchaseable upon exercise of this Warrant, or in the Exercise Price, the Company shall promptly compute such adjustment or readjustment in accordance with the provisions hereof and prepare a certificate showing such adjustment or readjustment, and shall mail such certificate to the holder of this Warrant in accordance with the notice provisions of Section 17. The certificate shall set forth such adjustment or readjustment and indicate the number of shares of Common Stock and the Exercise Price in effect after such adjustment or readjustment. The provisions of this Section 7(c) shall similarly apply to successive reclassifications, exchanges, combinations substitutions, replacements or other similar events.

8. No Fractional Shares or Scrip. No fractional shares or scrip representing fractional shares shall be issued upon the exercise of this Warrant, but in lieu of such fractional shares the Company shall make a cash payment therefor on the basis of the Exercise Price then in effect.

9. Reservation of Shares. During the period between the Effective Date and the Expiration Date, the Company shall at all times reserve and keep available out of its authorized but unissued shares of Common Stock or other securities constituting Shares, solely for the purpose of issuance upon the exercise of this Warrant, the maximum number of Shares issuable upon the exercise of this Warrant, and the par value per Share shall at all times be less than or equal to the applicable Exercise Price.

10. Representations of the Company. The Company represents that all corporate actions on the part of the Company, its officers, directors and stockholders necessary for the sale and issuance of this Warrant have been taken. The Company will pay all original issue and transfer taxes, if any, with respect to the issue and delivery of the Shares pursuant hereto and all other fees and expenses necessarily incurred by the Company in connection herewith.

11. Representations and Warranties by the Holder. The holder of this Warrant represents and warrants to the Company as follows:

    a. This Warrant and the Shares issuable upon exercise thereof are being acquired for its own account, for investment and not with a view to, or for resale in connection with, any distribution or public offering thereof within the meaning of the Securities Act of 1933, as amended (the "Act"). Upon exercise of this Warrant, the holder of this Warrant shall, if so requested by the Company, confirm in writing, in a form satisfactory to the Company, that the securities issuable upon exercise of this Warrant are being acquired for investment and not with a view toward distribution or resale.

    b. The holder of this Warrant understands that this Warrant and the Shares have not been registered under the Act by reason of their issuance in a transaction exempt from the registration and prospectus delivery requirements of the Act pursuant to Section 4(a)(2) thereof, and that they must be held by the holder indefinitely, and that the holder must therefore bear the economic risk of such investment indefinitely, unless a subsequent disposition thereof is registered under the Act or is exempted from such registration.

c. The holder of this Warrant has such knowledge and experience in financial and business matters that it is capable of evaluating the merits and risks of the purchase of this Warrant and the Shares purchasable pursuant to the terms of this Warrant and of protecting its interests in connection therewith.

d. The holder of this Warrant is aware of the Company's business affairs and financial condition and has received or has had full access to all the information it considers necessary or appropriate to make an informed investment decision with respect to the acquisition of this Warrant and its underlying securities. The holder of this Warrant further has had an opportunity to ask questions and receive answers from the Company regarding the terms and conditions of the offering of this Warrant and its underlying securities and to obtain additional information (to the extent the Company possessed such information or could acquire it without unreasonable effort or expense) necessary to verify any information furnished to the holder of this Warrant or to which the holder has access.

e. The holder of this Warrant is able to bear the economic risk of the purchase of the Shares pursuant to the terms of this Warrant.

f. The holder of this Warrant is an "accredited investor" as such term is defined in Rule 501 of Regulation D promulgated under the Act.

12. Restrictive Legend. The Shares shall be stamped or imprinted with a legend in substantially the following form:

THE SHARES REPRESENTED BY THIS CERTIFICATE HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF, AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED. SUCH SHARES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR AN EXEMPTION THEREFROM UNDER THE ACT AND ANY APPLICABLE STATE SECURITIES LAWS. COPIES OF THE AGREEMENT COVERING THE PURCHASE OF THESE SHARES AND RESTRICTING THEIR TRANSFER MAY BE OBTAINED AT NO COST BY WRITTEN REQUEST MADE BY THE HOLDER OF RECORD OF THIS CERTIFICATE TO THE SECRETARY OF THE COMPANY AT THE PRINCIPAL EXECUTIVE OFFICES OF THE COMPANY.

13. Warrants Transferable.

a. This Warrant, and any Shares issued hereunder, may not be sold, assigned or otherwise transferred by the holder hereof until six months after the Effective Date, except for an assignment or transfer of this Warrant, in whole or in part, by Neptune to an affiliate of Neptune, excluding any affiliate of the Company that is a natural person. Thereafter, this Warrant and the Shares may be offered, sold or otherwise transferred or disposed, in whole or in part, by the holder. Any sale, transfer or assignment of this Warrant or the Shares issued hereunder shall be subject to compliance with the terms and conditions of Section 13(b) and applicable federal and state securities laws.

b. Any transfer of this Warrant shall require surrender of this Warrant properly endorsed or accompanied by written instructions of transfer. With respect to any offer, sale or other disposition of this Warrant or the Shares, the holder hereof agrees to give written notice to the Company prior thereto, describing briefly the manner thereof, together with a written opinion of the holder's counsel, or other evidence, if requested by the Company, to the effect that such offer, sale or other disposition may be effected without registration or qualification (under the Act as then in effect or any federal or state securities law then in effect) of this Warrant or the Shares and indicating whether or not under the Act certificates for this Warrant or the Shares to be sold or otherwise disposed of require any restrictive legend as to applicable restrictions on transferability in order to ensure compliance with such law; provided, however, that (i) the Company shall not require the holder to provide an opinion of counsel if the transfer is to any affiliate of the holder, provided that any such transferee is an "accredited investor" as defined in Regulation D promulgated under the Act, and additionally, (ii) the Company shall also not require an opinion of counsel if there is no material question as to the availability of Rule 144 promulgated under the Act. Upon receiving such written notice and reasonably satisfactory opinion or other evidence, if so requested, the Company, as promptly as practicable, shall notify the holder that the holder may sell or otherwise dispose of this Warrant or the Shares, all in accordance with the terms of the notice delivered to the Company. If a determination has been made pursuant to this Section 13 that the opinion of counsel for the holder or other evidence is not reasonably satisfactory to the Company, the Company shall so notify the holder promptly with details thereof after such determination has been made. Each certificate representing this Warrant or Shares transferred in accordance with this Section 13 shall bear a legend as to the applicable restrictions on transferability in order to ensure compliance with such laws, unless in the aforesaid opinion of counsel for the holder, such legend is not required in order to ensure compliance with such laws. The Company may issue stop transfer instructions to its transfer agent in connection with such restrictions.

14. Reserved.

15. No Rights as Stockholder. The holder of this Warrant, as a holder of this Warrant, will not have any voting rights or other rights of a stockholder of the Company until exercise of this Warrant.

16. Expiration of Warrant. This Warrant shall be exercisable in whole or in part, at any time and from time to time, until ten years from the original issue date of this Warrant (the "Expiration Date"). If this Warrant has not been exercised prior to the Expiration Date, this Warrant shall be deemed to have been automatically exercised on the Expiration Date by "cashless" conversion pursuant to Section 4(a)(iii).

17. Notices. Any notice, consent, claim, demand, waiver, or other communication under this Warrant have legal effect only if in writing and addressed to a party to the address set forth below (or to such other address or such other person that a party may designate for itself from time to time in accordance with this Section). Notices sent in accordance with this Section will be deemed effectively given: (a) when received, if delivered by hand, with signed confirmation of receipt; (b) when received, if sent by a nationally recognized overnight courier, signature required; (c) when sent, if by email, (in each case, with confirmation of transmission), if sent during the addressee's normal business hours, and on the next business day, if sent after the addressee's normal business hours; and (d) on the third day after the date mailed by certified or registered mail, return receipt requested, postage prepaid.

Neptune:
c/o RTW Investments, LP
40 10th Avenue, Floor 7
New York, NY 10014
Attention: Legal Department

with a copy to Neptune's counsel:
Gibson, Dunn & Crutcher LLP
555 Mission Street
Suite 3000
San Francisco, CA 94105
Attention: Ryan A. Murr

Company:
Rocket Pharmaceuticals, Inc.
9 Cedar Brook Drive
Cranbury, NJ 08512
Attention: Chief Executive Officer

18. Governing Law. This Warrant and all actions arising out of or in connection with this Agreement shall be governed by and construed in accordance with the laws of the State of New York, without regard to the conflicts of law provisions of the State of New York or of any other state.

19. Rights and Obligations Survive Exercise of Warrant. Unless otherwise provided herein, the rights and obligations of the Company, of the holder of this Warrant and of the holder of the Shares issued upon exercise of this Warrant, shall survive the exercise of this Warrant.

[*Signature page follows*]

IN WITNESS WHEREOF, the Company has caused this Warrant to be executed by its officer thereunto duly authorized as of the date first above indicated.

ROCKET PHARMACEUTICALS, INC.

By: _____

Name: Gaurav Shah
Title: President & CEO

EXHIBIT A

NOTICE OF EXERCISE

TO: ROCKET PHARMACEUTICALS, INC.

Attention: Chief Executive Officer

1. The undersigned hereby elects to purchase_____Shares pursuant to the terms of the attached Warrant.

2. Method of Exercise (Please initial the applicable blank):

☐ The undersigned elects to exercise the attached Warrant by means of a cash payment, and tenders herewith payment in full for the purchase price of the shares being purchased, together with all applicable transfer taxes, if any.

☐ The undersigned elects to exercise the attached Warrant by means of the net exercise provisions of Section 4(a)(iii) of the Warrant.

3. Please issue a certificate or certificates representing said Shares in the name of the undersigned or in such other name as is specified below:

_____

(Name)

_____

(Address)

4. The undersigned hereby represents and warrants that all representations and warranties of the undersigned set forth in Section 11 of the attached Warrant (including Section 11(f) thereof) are true and correct as of the date hereof.

_____

(Signature)

_____

(Name)

_____

(Title)

_____

(Date)

EXHIBIT B

FORM OF TRANSFER

(To be signed only upon transfer of Warrant)

FOR VALUE RECEIVED, the undersigned hereby sells, assigns and transfers unto_____the right represented by the attached Warrant to purchase_____shares of Common Stock of Rocket Pharmaceuticals, Inc. to which the attached Warrant relates, and appoints_____Attorney to transfer such right on the books of_____, with full power of substitution in the premises.

Dated:_____

(Signature must conform in all respects to name of Holder as specified on the face of the Warrant)
Address:_____

Signed in the presence of:

_____

Exhibit 21.1

Subsidiaries of Rocket Pharmaceuticals, Inc.

	Subsidiary	Jurisdiction of Incorporation	Rocket Ownership
1.	Rocket Pharmaceuticals, Ltd.	Cayman Islands	100%
2.	Rocket Foundation, Inc.	Delaware	100%
3.	Spacecraft Seven, LLC	Delaware	100%

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statement of Rocket Pharmaceuticals, Inc. on Form S-3 (No 333-253756) and Form S-8 (Nos. 333-236946, 333-204501, 333-212308, 333-216892 and 333-223488) of our reports dated February 28, 2022, on our audits of the consolidated financial statements as of December 31, 2021 and 2020, and for each of the years in the three year period ended December 31, 2022, and the effectiveness of Rocket Pharmaceuticals, Inc.'s internal control over financial reporting as of December 31, 2021, which reports are included in this Annual Report on Form 10-K to be filed on or about February 28, 2022.

/s/ EisnerAmper LLP

EISNERAMPER LLP
Iselin, New Jersey
February 28, 2022

Exhibit 31.1

Certifications under Section 302

I, Gaurav Shah, MD, certify that:

1. I have reviewed this annual report on Form 10-K of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's Board of Directors (or persons performing the equivalent functions):
    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 28, 2022

/s/ Gaurav Shah, MD

Gaurav Shah, MD
President, Chief Executive Officer and Director
(Principal Executive Officer)

Exhibit 31.2

Certifications under Section 302

I, Carlos Garcia-Parada, certify that:

1. I have reviewed this annual report on Form 10-K of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's Board of Directors (or persons performing the equivalent functions):
    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 28, 2022

/s/ Carlos Garcia-Parada

Carlos Garcia-Parada
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the annual report on Form 10-K of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended December 31, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certify, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to their knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 28, 2022

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

Date: February 28, 2022

/s/ Carlos Garcia-Parada
Carlos Garcia-Parada
Chief Financial Officer
(Principal Financial Officer)

The foregoing certifications are not deemed filed with the Securities and Exchange Commission for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (Exchange Act), and are not to be incorporated by reference into any filing of Rocket Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Exchange Act, whether made before or after the date hereof, regardless of any general incorporation language in such filing.