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, 2019
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.)**
350 Fifth Avenue, Suite 7530 **New York, NY**	
(Address of Principal Executive Offices)	**10118** **(Zip Code)**

(646) 440-9100
(Registrant's Telephone Number, Including Area Code)

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

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.01 par value	**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 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 28, 2019 (the last business day of the registrant's most recently completed second fiscal quarter) was approximately $462.5 million, based upon the closing price on the Nasdaq Global Market reported for such date.

As of March 2, 2020, there were 55,071,194 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 2020 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, generic entrants, 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;
- 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.

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 pediatric diseases. We currently have three clinical-stage *ex vivo* lentiviral vector ("LVV") programs currently enrolling patients in the US and EU 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 US and EU. In addition, in the US 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. Finally, we have a pre-clinical stage LVV program for Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption – this program is anticipated to enter the clinic in 2020. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements. Additional work in the discovery stage for an FA CRISPR/CAS9 program as well as a gene therapy program for the less common FA subtypes C and G is ongoing.

Through our gene therapy platforms, we aim to restore normal cellular function by modifying the defective genes that cause each of the targeted disorders.

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.

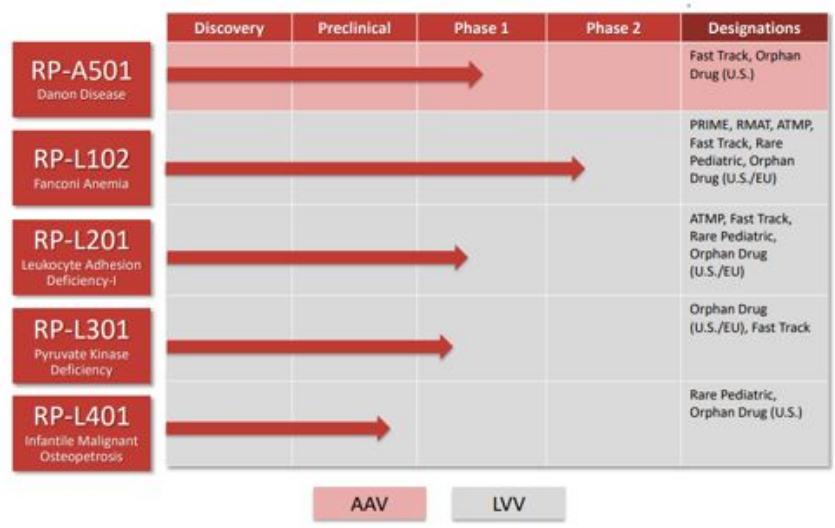
Essential Terminology.

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)	2.0 (“normal” value) 0.5 to 2 has been target in some LVV clinical studies (5.0 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 many disorders may be correctable with *in 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, PKD, and IMO.

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 lead our gene therapy programs into the next generation of promising therapy:

1. *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.

2. *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 demonstrates 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 (Nicoletti et al) demonstrates a correlation with lowered risk of both bone marrow failure and hematologic malignancy. This selective advantage also has been demonstrated by the results of the 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.

3. *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, 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.

Clinical Development Programs RP-L101 and RP-L102

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, both for the RP-L101 and RP-L102 programs. 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.

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 about 4,000, and given the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now thought to be in the 400 to 500 range.

We currently have one 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 sponsored Phase 2 registrational enabling clinical trials treating FA patients initially at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford") and Hospital Infantil de Nino Jesus ("HNJ") in Spain. The trial is expected to enroll ten patients total from the U.S. and EU. 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.

In October 2019, at the European Society of Cell and Gene Therapy ("ESGCT") 2019 Annual Congress, long-term Phase 1/2 clinical data of RP-L102, from the clinical trial sponsored by CIEMAT, for FA "Process A", without the use of myeloablative conditioning was presented demonstrating evidence of increasing and durable engraftment leading to bone marrow restoration exceeding the 10% threshold agreed to by the FDA and EMA for the ongoing registration-enabling Phase 2 trial. In one patient, who received what we consider adequate drug product, hemoglobin levels are now similar to those in the first year after birth, suggesting hematologic correction over the long term.

During the third quarter of 2019, we received alignment from the FDA on the trial design and the primary endpoint. This alignment was similar to that previously received from the European Medicines Agency ("EMA"). Resistance to mitomycin-C, a DNA damaging agent, in bone marrow stem cells at a minimum time point of one year is to serve as the primary endpoint for our currently ongoing Phase 2 study.

In December 2019, we announced that the first patient of the global Phase 2 study for RP-L102 "Process B" for FA received investigational therapy. There will be total of 10 patients enrolled in the global Phase 2 studies.

In December 2019, we also announced preliminary results from two pediatric patients treated with "Process B" RP-L102 prior to development of severe bone marrow failure in our Phase 1 trial of RP-L102 for FA. To evaluate transduction efficiency, an analysis of the proportion of the MMC-resistant colony forming cells was conducted and both patients have thus far exhibited early signs of engraftment, including increases in blood cell lineages in one patient. No drug-related safety or tolerability issues have been reported.

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 into tissues where these cells are needed to combat infections. As is the case with many rare diseases, true estimates of incidence are difficult; 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 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 is serving as the lead clinical site in Spain.

The ongoing open-label, single-arm, Phase 1/2 registration enabling clinical trial of RP-L201 has dosed one severe LAD-I patient in the U.S. to assess the safety and tolerability of RP-L201. The first patient was treated with RP-L201 in third quarter 2019. This study has received $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.

In December 2019, we announced initial results from the first pediatric patient treated with RP-L201, demonstrating early evidence of safety. Analyses of peripheral vector copy number ("VCN"), and CD18-expressing neutrophils were performed through three months after infusion of RP-L201 to evaluate engraftment and phenotypic correction. The patient exhibited early signs of engraftment with VCN myeloid levels at 1.5 at three months and CD-18 expression of 45%. No safety or tolerability issues related to RP-L201 administration (or investigational product) had been identified as of that date. The study is expected to enroll nine patients globally.

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.

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 1-3g/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 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. Rocket has 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 is likely 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.

Of note, proprietary transduction protocols in PKD now yield product VCNs of 2, with VCNs increasing to ≥ 4 with the addition of transduction enhancers. We expect that mobilization and harvesting procedures will be relatively straightforward for PKD patients.

Market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 per year.

Preclinical Proof-of-Concept

Preclinical results have demonstrated that RP-L301 corrects multiple components of the disorder in a PKD mouse model, including increases in hemoglobin (in both primary and secondary transplant recipients), reduction in reticulocytosis, which is an increase in immature red blood cell production, correction of splenomegaly and reduction in hepatic erythroid clusters and iron deposits.

Our Clinical Trial & Regulatory Status

We currently have one 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 initial global Phase 1 study cleared in October 2019. This program has been granted EMA orphan drug disease designation and FDA orphan drug disease designation ("ODD").

This global Phase 1 open-label, single-arm, clinical trial will enroll six adult and pediatric transfusion-dependent PKD patients in the U.S. and Europe. Lucile Packard Children's Hospital Stanford will serve as the lead site in the U.S. for adult and pediatric patients, and Hospital Infantil Universitario Niño Jesús 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.

Enrollment for the Phase 1 clinical trial of RP-L301 is currently ongoing at Stanford and the University Hospital Fundacion Jimenez Diaz ("FJD") and preliminary data is anticipated in the second half of 2020.

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. Normally, 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.

IMO is characterized by increased bone mass and density, multiple deformities and a propensity for fractures in patients surviving infancy. Skull deformities include macrocephaly and frontal bossing. Thoracic size may be decreased. Bone sclerosis impinges cranial nerve and spinal foramina with resulting neurologic abnormalities, including hydrocephalus, progressive blindness and auditory impairment. Compression of bone marrow space results in bone marrow failure with compensatory hepatosplenomegaly and increased infection risk secondary to neutropenia.

Current Therapy

Allogeneic HSCT is potentially curative, but notable for considerable rates of engraftment failure, GVHD and pulmonary and hepatic complications. In a recent multicenter retrospective series, long-term survival rates for HSCT recipients with IMO were approximately 60% for matched-sibling recipients, and 40% for those with mismatched or unrelated allografts.

Preclinical Proof-of-Concept

Because osteoclasts are derived from the monocyte/macrophage lineage, correction of the TCIRG1 gene in hematopoietic stem cells will enable development of functional, bone-resorbing osteoclasts, as has been demonstrated in preclinical models. Preclinical results demonstrate that gene correction of HSPCs from IMO patients is feasible, and that these HSPCs can engraft in immunocompromised mice. Osteoclasts from these mice demonstrate increased bone resorption in vitro, as measured by increased calcium and collagen fragment CTX-I.

Additional preclinical experiments have demonstrated correction of an osteopetrotic (IMO) phenotype displayed by the oc/oc mouse model, in which even limited engraftment of wild-type murine bone marrow cells (including 4 to 5% wild-type engraftment) has been associated with reversal of the osteopetrosis phenotype.

Regulatory Status

We currently have one LVV-based program targeting IMO, RP-L401. RP-L401 is a preclinical program that we in-licensed from Lund University, Sweden. This program has been granted ODD and Rare Pediatric Disease designation from the FDA. The FDA defines a "rare pediatric disease" as a serious and life-threatening disease that affects less than 200,000 people in the U.S. that are aged between birth to 18 years. The Rare Pediatric Disease designation program allows for a sponsor who receives an approval for a product to potentially qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. We have partnered with UCLA to lead U.S. clinical development efforts for the IMO program and anticipate that UCLA will serve as the lead U.S. clinical site for IMO. We intend to file an IND for IMO and commence our clinical trial in the second half of 2020.

AAV Program.

Overview of Danon Disease

Danon disease is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. Danon disease 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 Danon disease symptoms can include skeletal muscle weakness, liver disease, and intellectual impairment. There are no specific

therapies available for the treatment of Danon disease. 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, however new market research is being performed and the prevalence of patients may be updated in the future.

In January 2019, we announced the clearance of our IND application by the FDA for RP-A501, and in February 2019, we were notified by the FDA that we were granted Fast Track designation for RP-A501. University of California San Diego Health is the initial and lead center for our Phase 1 clinical trial.

On May 2, 2019, we presented additional preclinical data at the ASCGT annual meeting, indicating that high VCN, in Danon disease-relevant organs in both mice and non-human primates ("NHN's"), with high concentrations in heart and liver tissue (for NHP, cardiac VCN was approximately 10 times higher on average than in skeletal muscle and central nervous system), which is consistent with reported results in several studies of heart tissue across different species. There were no treatment-related adverse events or safety issues up to the highest dose. We have dosed three patients in the RP-A501 phase 1 clinical trial. We will continue further enrollment with clinical data read-outs in the second half of 2020.

CRISPR/Cas9 gene editing in Fanconi Anemia:

In addition to its LVV and AAV programs, Rocket also has a program evaluating CRISPR/Cas9-based gene editing for FA. This program is currently in the discovery phase. CRISPR/Cas9-based gene editing is a different method of correcting the defective genes in a patient, where the editing is very specific and targeted to a particular gene sequence. "CRISPR/Cas9" stands for Clustered, Regularly Interspaced Short Palindromic Repeats ("CRISPR") Associated protein-9. The CRISPR/Cas9 technology can be used to make "cuts" in DNA at specific sites of targeted genes, making it potentially more precise in delivering gene therapies than traditional vector-based delivery approaches. CRISPR/Cas9 can also be adapted to regulate the activity of an existing gene without modifying the actual DNA sequence, which is referred to as gene regulation.

Regulatory Status

This program is currently in the discovery stage of drug development.

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 commercialization.

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 in-licensed numerous 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 March 2, 2020, 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 patent 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. Rocket expects 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 pending PCT application with claims directed to gene therapy vectors for the treatment of Danon disease. Any patents, if issued, arising from any national stage applications filed from this PCT application, 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.

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 files, 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 Fred Hutchinson Cancer Research Center ("Hutch")

In November 2015, we entered into an exclusive license agreement with Hutch granting us worldwide, sublicensable, exclusive rights to certain patents, materials and other intellectual property relating to lentiviral vector-based technology for patient stem cell transduction useful for, among other things, treating Fanconi Anemia.

We are obligated to make aggregate cash milestone payments of up to $1.6 million to Hutch 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 low to mid-single digit percentage royalty on net sales, subject to specified adjustments, by us or its 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 sublicenses in specified circumstances.

In December 2015, we entered into an exclusive license agreement with Hutch granting us worldwide, sublicensable, exclusive rights to certain patents covering Hutch's "Prodigy" platform, a portable platform for hematopoietic stem/progenitor cell gene therapy.

As consideration for the licensed rights, in January 2016 we issued to Hutch ordinary shares valued at $0.1 million as an upfront license that was expensed as research and development ("R&D") costs. We are obligated to make aggregate milestone payments of up to $0.2 million, which may include amounts already paid, to Hutch upon the achievement of specified development and regulatory milestones. 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 sublicenses in specified circumstances.

We may terminate this agreement at any time by providing Hutch with 180 days advance notice. The agreement will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in all countries worldwide.

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 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 creates.

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 Rocket enters 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.

Contract Research and Collaboration Agreement with Lund University and J. Richter

In August 2016, we entered into a research and collaboration agreement with Lund University and Johan Richter, M.D., Ph.D. under which Dr. Richter granted to us an exclusive, perpetual, sublicensable, worldwide license to certain intellectual property rights of Dr. Richter relating to lentiviral-mediated gene transfer to treat IMO. In exchange for the license, we are 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) and sublicense revenue payments to Dr. Richter. Under the terms of the agreement, Lund University and Dr. Richter are obligated to perform contract research for us regarding the use of lentiviral-mediated gene transfer to treat IMO. Intellectual property resulting from the contract research created by Dr. Richter is included in the license described above and also subject to an option for us to purchase ownership of such rights. Intellectual property created by Lund University in conducting such research is non-exclusively licensed to us for non-commercial use and also subject to an option for us to purchase or license such intellectual property under commercially reasonable terms. We are obligated to pay for the contract research according to an agreed budget in quarterly installments in advance.

As consideration for an option to acquire rights from Lund University on commercially reasonable terms and conditions, we paid Lund University an upfront license fee of €0.02 million (approximately $0.02 million), which was expensed as R&D costs. We are obligated to make aggregate milestone payments of up to €0.1 million (approximately $0.1 million) to Lund University and Dr. Richter upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the Lund University agreement, we are obligated to pay a low 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.

The research and collaboration agreement had an initial term of 24 months and in August 2018, was amended for an additional year expiring August 2019 and amended in August 2019, amended for an additional year expiring August 2020.

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 as R&D costs. 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 through our wholly-owned subsidiary Rocket Pharmaceuticals, Ltd., 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.

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 anticipates 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 do not currently operate manufacturing facilities for clinical or commercial production of our product candidates, although we anticipate beginning technology transfer activities at our facility in Cranbury, New Jersey in 2020. Beginning in 2021, we plan to supplement current supply arrangements with our own direct manufacturing capabilities for our AAV 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 through the biologics, including its gene therapy product candidate BLA process, 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;
- 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 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.

Where a gene therapy trial is conducted at, or sponsored by, institutions receiving National Institutes of Health ("NIH"), funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the study is registered with the NIH Office of Biotechnology Activities, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules ("NIH Guidelines"). Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA; however, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

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, an IRB, a data safety monitoring board 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.

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. Each BLA submission requires a substantial user fee payment unless a waiver or exemption applies. 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. 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. 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. 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 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

Market exclusivity provisions under the FDCA can delay the submission or the approval of certain competing applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the U.S. to the first applicant to gain approval of a BLA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, with certain exceptions, the FDA may not accept for review a BLA submitted by another company for a drug product that contains the protected active moiety.

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.

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 breakthrough therapy designation, priority review and accelerated approval.

- *Regenerative medicine advanced therapy (RMAT) designation*. To qualify for the RMAT program, product 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 drug has the potential to address unmet medical needs for such disease or condition.

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

- *Accelerated approval*. Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, 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 as a condition for accelerated approval pre-approval of promotional materials.

Fast Track designation, breakthrough therapy designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

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

Post-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. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our LVV products in accordance with cGMP regulations. We anticipate that we will also utilize direct manufacturing capabilities for clinical supplies for our AAV program, beginning in 2021. 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. 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 sponsor under 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. This statute has been interpreted to prohibit presenting claims for items or services not provided as claimed, or claims for medically unnecessary items or services. 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 of between $5,500 and $11,000 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. 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 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.

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.

European Union Drug Development

In the EU, our products will also be subject to extensive regulatory requirements. As in the United States, medicinal products can only be marketed if a marketing authorization application ("MAA") from the competent regulatory agencies has been obtained, and the various phases of preclinical and clinical research in the EU are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC (the "EU Clinical Trials Directive") has sought to harmonize the EU clinical trial regulatory framework, setting out common rules for the control and authorization of clinical trials in the EU, the EU Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved by two distinct bodies in each of the EU countries where the trial is to be conducted: the National Competent Authority ("NCA") and one or more Ethics Committees ("ECs"). In addition, all serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the NCA and ECs of the Member State where they occurred.

The EU Clinical Trial Regulation (Regulation (EU) No 536/2014) (the "EU Clinical Trial Regulation") will repeal the EU Clinical Trials Directive when it becomes applicable, which is expected to occur in 2020. The EU Clinical Trial Regulation is aimed at making more uniform and streamlining the clinical trials authorization process, simplifying adverse event reporting procedures, improving the supervision of clinical trials and increasing the transparency of clinical trials.

European Union Drug Review Approval

In the European Economic Area ("EEA"), which is comprised of the 28 Member States of the EU plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining an MAA. There are two types of MAAs: (1) the Community MAA, which is issued by the European Commission through the Centralized Procedure based on the opinion of the Committee for Medicinal Products for Human Use, a body of the EMA, and which is valid throughout the entire territory of the EEA; and (2) the National MAA, which is issued by the competent authorities of the Member States of the EEA and only authorized 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 and medicinal products containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU. The National MAA is 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 EEA, this National MAA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MAA 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 the MAA 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 MAA in all the Member States where the authorization was sought. Before granting the MAA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

In addition, in the EU, the EMA's CAT is responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. 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.

Employees

We had 57 full-time employees as of March 2, 2020, none of our employees are represented by any collective bargaining unit. We believe that we maintain good relations with our employees.

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 The Empire State Building, 350 Fifth Avenue, Suite 7530, New York, NY 10118, 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, 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.

Risks Related to Our Financial Position

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 effort, 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 $77.3 million and $74.5 million for the years ended December 31, 2019 and 2018, respectively. As of December 31, 2019, we had an accumulated deficit of $183.1 million. Substantially all of our operating losses have resulted from costs incurred in connection with our R&D programs 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 testing, 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 incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our accumulated deficit and working capital.

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 seek to broaden licensing of our gene therapy platforms, complete 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 trials, and expect to spend up to $30.0 million in non-recurring expenses in 2020 in connection with the buildout of our new facility in Cranbury, New Jersey. Also, if we obtain marketing approval for any of our 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, including with our transition from emerging growth company and expected transition from smaller reporting company status at the end of 2020. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations, and we may seek to refinance or exchange our convertible notes to further extend their maturity date. If we are unable to raise capital, or refinance our convertible notes due 2022 when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our licensing activities, our R&D programs or other operations. Furthermore, to the extent we raise additional capital by issuing equity securities, or to the extent holders of our 2022 convertible notes exercise their option to convert their notes into shares of our common stock, our stockholders will experience substantial additional dilution.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2019, our cash, cash equivalents and investments was $304.1 million. Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement, completion and results of our clinical trials;
- the production of LVV and AAV gene therapy products 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 drug discovery, laboratory testing, preclinical development and clinical trials, if any, for our internal product candidates; the costs associated with building out additional laboratory and research capacity;
- 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 preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs associated with being a public company.

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.

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.

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 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 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 Product Regulatory Matters

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, 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.

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.

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 the patients in the studies;
- political unrest at 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. Additionally, the process of finding 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. 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.

We have initiated our 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. Our other gene therapy program for IMO is in the preclinical stage though we plan to submit an IND and commence clinical trials in the second half of 2020. 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.

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.

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 REMS. 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. 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, our products will remain subject to regulatory scrutiny.

Even if we obtain regulatory approval in a jurisdiction, 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. 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. 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 United States 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.

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.

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 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, 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 Manufacturing, Development and Commercialization of 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.

We may encounter problems contracting with, hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or 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.

We are in the process of completing the build-out of a manufacturing site that could support future production of our product candidates. We have no experience in manufacturing, and there can be no assurance that we will be able to complete our manufacturing facility or, if completed, we will be able to successfully manufacture products.

We have historically relied on third parties to manufacture supplies of our product candidates. We are now in the process of building a manufacturing facility in Cranbury, New Jersey, which we expect to be complete in the first half of 2020. Designing and building a manufacturing facility is time-consuming, expensive, and may be subject to delays or cost overruns.

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.

Even if approved, we may not successfully commercialize our drug 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 drug 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.

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

Market acceptance and sales of our product candidates may depend on coverage and reimbursement policies and health care reform measures. Decisions about formulary coverage as well as levels at which government authorities and third-party payors, such as private health insurers and health maintenance organizations, reimburse patients for the price they pay for our products as well as levels at which these payors pay directly for our products, where applicable, could affect whether we are able to successfully commercialize these products. We cannot guarantee that reimbursement will be available for any of our product candidates, nor can we guarantee that coverage or reimbursement amounts will not reduce the demand for, or the price 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. 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. 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.

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.

We may not be successful in our efforts to build a pipeline of additional product candidates.

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 R&D efforts to date have resulted in. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. If we do not successfully develop and commercialize product candidates based upon our approach, 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 of time. R&D was our most significant operating expense for the year ended December 31, 2019. 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 on 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.

The commercial success of any of our product candidates will depend upon our 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 these 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, in particular, 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. The failure of any of our product candidates to achieve market acceptance could materially harm our business, financial condition, results of operations and prospects.

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 products 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.

Risks Related to Personnel and Other Risks Related to Our Business

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; and our Chief Operating Officer and Head of Development, Kinnari Patel, PharmD, MBA. 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.

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

As of March 2, 2020, we had 57 full-time employees. 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.

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 in the course of 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.

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.

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.

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.

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.

In the United States, there have been and continue to be legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or the ACA, was passed, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. Since January 2017, the Trump administration has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One Executive Order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. This decision was appealed to the U.S. Supreme Court, and oral arguments were heard in December 2019. The effects of the gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known.

Since its enactment, some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial, congressional, or executive challenges. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the ACA. The U.S. Supreme Court has upheld certain key aspects of the legislation, including a tax-based shared responsibility payment imposed on certain individuals who fail to maintain qualifying health coverage for all or part of a year or pay a penalty, which is commonly known as the “individual mandate.” However, as a result of tax reform legislation passed in December 2017, the individual mandate was eliminated effective January 1, 2019. On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans and the annual fee imposed on certain health insurance providers based on market share. The Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.”

In July 2018, the U.S. Centers for Medicare and Medicaid Services ("CMS") published a final rule permitting further collections and payments to and from certain ACA-qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS published a final rule that gave states greater flexibility, starting in 2020, 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.

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 in spending reductions. 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, on average, 2% per fiscal year through 2025 unless Congress takes additional action. These reductions were extended through 2027 under the BBA. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further 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.

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. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services, or HHS, has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020. The Trump administration has also proposed a program that, if finalized, would permit the importation of lower-cost drugs from other countries, including Canada. Although a number of these, and other proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will 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.

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.

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 U.K. ceased being a Member State of the EU on January 31, 2020. However, the terms of the withdrawal have yet to be fully negotiated. The implementation period began February 1, 2020 and will continue until December 31, 2020. During this 11-month period, the U.K. will continue to follow all of the EU's rules, the EU's pharmaceutical law remains applicable to the U.K. and the U.K.'s trading relationship will remain the same. However, regulations (including financial laws and regulations, tax and free trade agreements, intellectual property rights, data protection laws, supply chain logistics, environmental, health and safety laws and regulations medicine licensing and regulations, immigration laws and employment laws), have yet to be addressed. This has caused political and economic uncertainty, including in the regulatory framework applicable to our operations and potential products, and this uncertainty may persist for years. Brexit could, among other outcomes, disrupt the free movement of goods, services and people between the United Kingdom and the EU, and result in increased legal and regulatory complexities, as well as potential higher costs of conducting business in Europe. The transition of the United Kingdom from the EU may cause disruption or delays in granting clinical trial authorization or opinions for marketing authorization, disruption of importation and export of active substance and other components of new drug formulations, and disruption of the supply chain for clinical trial product and final authorized formulations.

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.

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. has adopted the General Data Protection Regulation ("GDPR"), which introduces strict requirements for processing personal data. The GDPR is likely to increase 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 physical health conditions, may impose 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.

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 each of these 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.

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.

We or the third parties upon whom we depend may be adversely affected by natural disasters and/or health epidemics, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations or the operations of our third parties, including our third-party contract manufacturers, our CROs and contract laboratories or our strategic partners, and have a material adverse effect on our business, results of operations, financial condition and prospects. If natural disasters, such as earthquakes, tsunamis, power shortages or outages, floods or monsoons, public health crises, such as pandemics and epidemics, political crises, such as terrorism, war, political instability or other conflict, cyberattacks, or other events outside of our control occurred that prevented us or third parties from using all or a significant portion of our or their offices, manufacturing and/or laboratory spaces, that damaged critical infrastructure, such as manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us or third parties upon which we rely to continue our or their business and operations for a substantial period of time.

Our business could be adversely impacted by the effects of the coronavirus (COVID-19) outbreak originating in China in December 2019, or by other epidemics and pandemics. We currently rely on third-party manufacturers to produce the plasmids, vectors, cell banks and final drug product for our clinical trials, and though we do not currently source our materials from China, new quarantines for COVID-19 or other viruses could impact personnel at our or third party manufacturing facilities in the United States and other countries or the availability or cost of materials, which would disrupt our supply chain. In addition, certain of our research and development efforts are conducted globally. Due to the impact of COVID-19, our employees or third-parties on which we rely could be quarantined, and healthcare resources could be diverted from our clinical trials. A health epidemic or pandemic or other outbreak, including the current COVID-19 outbreak, could materially and adversely affect our business, financial condition and results of operations.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Risks Related to Our Intellectual Property

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

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

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

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

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.

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.

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. For example, Institute 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 be entirely expired by 2023, if our products were to launch before 2023, we may need to secure a license.

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.

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.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we 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 would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have 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.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer it a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

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.

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. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act (the Leahy-Smith Act), signed into law on September 16, 2011, could increase those uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter parties review, and derivation proceedings. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first inventor to file system in which, assuming that the other statutory requirements are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, 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.

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 31.9% 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.

If we are unable to implement and maintain effective internal control over financial reporting in the future, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our common stock may decline.

As a public company, we will be required to maintain internal control over financial reporting and to report any material weaknesses in such internal control. Once we are no longer an emerging growth company, 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. We are in the process of designing, implementing, and testing the internal control over financial reporting required to comply with this obligation, which process is time consuming, costly, and complicated. In addition, our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting beginning with our Annual Report following the date on which we are no longer an "emerging growth company." If we identify material weaknesses in our internal control over financial reporting, if we are unable to comply with the requirements of Section 404 in a timely manner or assert that our internal control over financial reporting is effective, or if our independent registered public accounting firm is unable to express an opinion as to the effectiveness of our internal control over financial reporting when required, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our common stock could be negatively affected, and we could become subject to investigations by the stock exchange on which our securities are listed, the Securities and Exchange Commission, or the SEC, or other regulatory authorities, which could require additional financial and management resources.

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.

We are an "emerging growth company" and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We will cease to be an emerging growth company as of December 31, 2020. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Item 1B. Unresolved SEC Comments

None.

Item 2. Properties

Corporate Headquarters

Our corporate headquarters is located in New York, New York at the Empire State Building, and consists of approximately 6,600 square feet of leased office space under a lease that expires in July 2021.

Laboratory Leases

We lease approximately 2,000 square feet of laboratory space at the Alexandria Center for Life Science in New York, New York with a term ending in July 2022.

Rocket has entered into 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 will be dedicated to AAV Current Good Manufacturing Practice (cGMP) manufacturing to support the Company's pipeline, which has not yet commenced. 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.

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 Select Market under the symbol "RCKT". Effective December 24, 2018, we began trading on the Nasdaq Biotechnology Index (Nasdaq: NBI).

Stockholders

As of March 2, 2020, there were 15 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay cash dividends will be made at the discretion of our board of directors. 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 quarter ended December 31, 2019.

Item 6. Selected Financial Data

Non-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.

Introduction

Rocket Pharmaceuticals, Inc., together with its subsidiaries (collectively, "Rocket" or the "Company"), is a clinical-stage, multi-platform biotechnology company focused on the development of first or best-in-class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating pediatric diseases. The Company has clinical-stage lentiviral vector ("LVV") programs currently undergoing clinical testing 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. FA has been in clinical stage testing in the European Union ("EU") since 2016, and in the United States ("U.S.") since the first quarter of 2019. Rocket received investigational new drug ("IND") clearance for both FA and LAD-I in late 2018 and PKD in the third quarter of 2019. Rocket also has a pre-clinical stage LVV program for Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. In addition, the Company has a clinical stage adeno-associated virus ("AAV"), program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The Company has global commercialization and development rights to all of its product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program for which the Company currently only has development rights.

Recent Developments

On April 18, 2019, we completed a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 675,000 shares of our common stock, at a public offering price of $17.50 per share. The gross proceeds to Rocket from the public offering were approximately $90.6 million, after deducting $4.5 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $86.1 million.

On April 30, 2019, the 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 I/II 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. On July 1, 2019, we received the first grant from CIRM of $0.8 million and on October 15, 2019, we received the second grant of $0.4 million, based on eligible costs incurred under the grant.

On December 10, 2019, we completed a public offering of 4,393,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 573,000 shares of our common stock, at a public offering price of $22.25 per share. The gross proceeds to us from the public offering were approximately $97.7 million, after deducting $6.1 million of offering costs, commissions, legal and other expenses for net gross proceeds from the offering of $91.7 million.

On February 10, 2020, we entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of our outstanding 5.75% Convertible Senior Notes due 2021 (the "Old Notes") to extend the maturity date by one year. Pursuant to the Exchange Agreements, we exchanged approximately $39.4 million aggregate principal amount of the Old Notes (which represents approximately 76% of the aggregate outstanding principal amount of the Old Notes) for (a) approximately $39.4 million aggregate principal amount of 6.25% Convertible Senior Notes due 2022 (the "New Notes") (an exchange ratio equal to 1.00 New Notes per exchanged Old Notes) and (b) $119,416 in cash to pay the accrued and unpaid interest on the exchanged Old Notes from, and including, February 1, 2020, to, but excluding, the closing date of the exchange transactions. The New Notes were issued in private placements exempt from registration in reliance on Section 4(a) (2) of the Securities Act of 1933, as amended (the "Securities Act"). Upon completion of the exchange transactions, approximately $12.7 million aggregate principal amount of Old Notes remained outstanding. The exchange transactions closed on February 20, 2020.

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, 2019, we raised net cash proceeds of approximately $373.1 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 R&D activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development. As a result, we expect that R&D expenses will increase substantially over the next several years as we increase personnel costs, including stock-based compensation, support ongoing clinical studies, seek to achieve proof-of-concept in one or more product candidates, advance preclinical programs to clinical programs, and prepare regulatory filings for product candidates.

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 its 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 Rocket's 2021 Convertible Notes, which mature in August 2021, a substantial amount of which were exchanged in February 2020 for 6.25% Convertible Senior Notes due in 2022.

Interest Income

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

Results of Operations

Comparison of the Years Ended December 31, 2019 and 2018

The following table summarizes our results of operations for the years ended December 31, 2019 and 2018:

(in thousands)

	For the Years Ended December 31,		
	2019	2018	Change
Operating expenses:			
Research and development	$ 58,623	$ 53,270	$ 5,353
General and administrative	17,528	17,886	(358)
Total operating expenses	76,151	71,156	4,995
Loss from operations	(76,151)	(71,156)	(4,995)
Research and development incentives	250	186	64
Interest expense	(5,958)	(6,039)	81
Interest and other income net	3,414	1,690	1,724
Accretion of discount on investments	1,175	801	374
Total other income (expense), net	(1,119)	(3,362)	2,243
Net loss	$ (77,270)	$ (74,518)	$ (2,752)

Research and Development Expenses

R&D expenses increased $5.4 million to $58.6 million for the year ended December 31, 2019 compared to the year ended December 31, 2018. The increase in R&D expenses was primarily a result of increases in clinical trial expenses of $6.3 million, an increase in compensation expense of $2.9 million due to increased R&D headcount, an increase in research agreements of $1.7 million, offset by a decrease in license expense of $5.6 million primarily due to the $7.0 million REGENXBIO Inc. license fee paid in 2018 which did not reoccur in 2019.

General and Administrative Expenses

G&A expenses decreased $0.4 million to $17.5 million for the year ended December 31, 2019 compared to the year ended December 31, 2018. Rocket incurred certain one-time stock compensation and severance termination expenses in Q-1, 2018 of $5.3 million related to the reverse merger with Inotek. Excluding the $5.3 million one-time reverse merger related G&A expenses incurred in 2018, G&A expenses would have increased $5.0 million primarily due to an increase in stock compensation expense of $4.5 million and G&A compensation expense of $1.0 million, offset by a decrease in legal fees of $0.5 million.

Other Income (Expense), Net

Other income (expense), net was $1.1 million for the year ended December 31, 2019 compared to other income (expense), net of $3.4 million for the year ended December 31, 2018. The change was primarily due to an increase in interest income of $1.7 million and an increase in accretion income of $0.4 million. The increase in interest income for the year ended December 31, 2019, was due to the Company's investment portfolio which increased primarily due to the proceeds from financings and higher interest rates reflected in the first half of 2019.

Liquidity and Capital Resources

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 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.

The Company's drug candidates are in the development and clinical stage. There can be no assurance that the Company's R&D 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. Rocket has incurred net losses and negative cash flows from its operations each year since inception. Rocket incurred net losses of $77.3 million and $74.5 million for the years ended December 31, 2019 and 2018, respectively. The Company has experienced negative cash flows from operations and has an accumulated deficit of $183.1 million as of December 31, 2019. As of December 31, 2019, the Company has $304.1 million of cash, cash equivalents and investments. Rocket expects such resources would be sufficient to fund its operating expenses and capital expenditure requirements into 2022. Rocket has funded its operations primarily through the sale of its equity and debt securities.

On April 18, 2019, the Company completed a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 675,000 shares of our common stock, at a public offering price of $17.50 per share. The net proceeds to Rocket from the public offering were approximately $86.1 million, after deducting $4.5 million of offering costs, commissions, legal and other expenses from the gross proceeds from the offering of $90.6 million.

On April 30, 2019, CIRM awarded Rocket 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 I/II 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. On July 1, 2019, we received the first grant from CIRM of $0.8 million and on October 15, 2019, we received the second grant of $0.4 million, based on eligible costs incurred under the grant.

On December 10, 2019, the Company completed a public offering of 4,393,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 573,000 shares of our common stock, at a public offering price of $22.25 per share. The net proceeds to Rocket from the public offering were approximately $91.7 million, after deducting $6.1 million of offering costs, commissions, legal and other expenses from the gross proceeds from the offering of $97.7 million.

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. 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. 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.

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 the Company (the "2021 Convertible Notes"). The 2021 Convertible Notes were issued in 2016 and mature on August 1, 2021 ("Maturity Date"). The 2021 Convertible Notes are unsecured, accrue interest at a rate of 5.75% per annum and interest is payable semi-annually on February 1 and August 1 of each year. Each holder of a 2021 Convertible Note (the "Holder") has 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 principal amount of 2021 Convertible Notes (the "Conversion Rate") which is $32.08 per share. The Conversion Rate is subject to adjustment from time to time upon the occurrence of certain events, including the issuance of stock dividends and payment of cash dividends.

On February 10, 2020, we entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of our outstanding 5.75% Convertible Senior Notes due 2021 (the "Old Notes"). Pursuant to the Exchange Agreements, we exchanged approximately $39.4 million aggregate principal amount of the Old Notes (which represents approximately 76% of the aggregate outstanding principal amount of the Old Notes) for (a) approximately $39.4 million aggregate principal amount of 6.25% Convertible Senior Notes due 2022 (the "New Notes") (an exchange ratio equal to 1.00 New Notes per exchanged Old Note) and (b) $119,416 in cash to pay the accrued and unpaid interest on the exchanged Old Notes from, and including, February 1, 2020, to, but excluding, the closing date of the exchange transactions. The New Notes were issued in private placements exempt from registration in reliance on Section 4(a) (2) of the Securities Act. Upon completion of the exchange transactions, approximately $12.7 million aggregate principal amount of Old Notes remained outstanding. The exchange transactions closed on February 20, 2020.

The conversion rate for the New Notes will initially be 31.1876 shares of the Company's common stock per $1,000 principal amount of 2022 Notes, which is equivalent to an initial conversion price of approximately $32.06 per share of common stock, and is subject to adjustment under the terms of the New Notes. The Company may redeem for cash all or any portion of the 2022 Notes, at its option, if the last reported sale price of its common stock is equal to or greater than 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending within the five trading days immediately preceding the date on which the Company provides written notice of redemption. The 2022 Notes Indenture contains customary terms and covenants and events of default.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

(in thousands)

	For the Years Ended December 31,	
	2019	**2018**
Cash used in operating activities	$ (64,663)	$ (53,788)
Cash used in investing activities	(39,011)	(5,272)
Cash provided by financing activities	177,791	153,502
Net change in cash, cash equivalents and restricted cash	$ 74,117	$ 94,442

Net Cash Used in Operating Activities

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 Rocket's 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.

During the year ended December 31, 2018, operating activities used $53.8 million of cash, primarily resulting from our net loss of $74.5 million and net changes in our operating assets and liabilities of $4.4 million, partially offset by net non-cash charges of $16.3 million, including stock-based compensation expense of $13.6 million and accretion of discount on convertible notes of $3.1 million. Changes in Rocket's operating assets and liabilities for the year ended December 31, 2018 consisted of an increase in accounts payable and accrued expenses of $5.9 million, offset by an increase in prepaid expenses and other assets of $1.5 million.

Net Cash Used in Investing Activities

During the year ended December 31, 2019, net cash outflow of 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.

During the year ended December 31, 2018, net cash outflow of investing activities was $5.3 million, consisting primarily of investment purchases of $141.1 million and purchases of property and equipment of $1.5 million, offset by $76.3 million of cash acquired in connection with the reverse merger, and $61.3 million from the maturities of investments.

Net Cash Provided by Financing Activities

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, the Company 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.

During the year ended December 31, 2018, net cash provided by financing activities was $153.5 million, consisting primarily of proceeds from the issuance of common stock. On January 26, 2018, we completed a public offering of common stock and received net proceeds of approximately $78.5 million. On November 30, 2018, we completed a public offering of common stock and received net proceeds of $74.1 million.

Off-Balance Sheet Arrangements

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

JOBS Act

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We will cease to be an emerging growth company as of December 31, 2020.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Critical Accounting Policies and Estimates

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. We have one segment and one reporting unit and as such reviews goodwill for impairment at the consolidated level.

When testing goodwill, we have 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. We may also consider 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.

In January 2017, the FASB issued ASU No. 2017-04, "Intangibles- Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment", an amendment to simplify the subsequent quantitative measurement of goodwill by eliminating step two from the goodwill impairment test. We early adopted this guidance as of January 1, 2018. An entity will recognize an impairment charge for the amount by which the carrying amount of a reporting unit exceeds its fair value, not to exceed the total amount of goodwill allocated to the reporting unit. We performed the qualitative assessment of Rocket's 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, we have determined there was no goodwill impairment for the years ended December 31, 2019 and 2018.

Impairment of Long-Lived Assets

We review long-lived assets for impairment whenever events or changes in circumstances indicate that the asset's carrying amount may not be recoverable. We 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.

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.

Fair Value Measurements

We are 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. Accounting Standard Codification (“ASC”) Topic 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of our company. Unobservable inputs are inputs that reflect our 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 we have 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 our 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 us 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. The fair value of the 2021 Convertible Notes as of December 31, 2019 was approximately $54.5 million. The Company’s assets and liabilities measured at fair value on a recurring basis include its short and long term investments. 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 Government, Corporate and Municipal 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.

Stock-Based Compensation

We measure the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which the employee 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. Our estimates of these assumptions are primarily based on historical data, peer company data and judgment regarding future trends and factors.

Recent Accounting Pronouncements

The Company adopted ASU 2016-02, Leases ("ASU 2016-02"), as amended, on January 1, 2019, which supersedes the prior leasing guidance and upon adoption, requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet for all leases with terms longer than 12 months. Leases are classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Upon the adoption of the guidance, operating leases were capitalized on the balance sheet at the present value of lease payments. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 was calculated using an estimate of the Company's collateralized borrowing rate for debt with a similar term.

In adopting ASU 2016-02, the Company elected the available package of practical expedients which allows the Company to not reassess previous accounting conclusions around whether arrangements are or contain leases, the classification of leases, and the treatment of initial direct costs. The Company also made an accounting policy election to utilize the short-term lease exemption, whereby leases with a term of 12 months or less will not follow the recognition and measurement requirements of the new standard. Upon adoption in 2019, the Company recognized total right-of-use assets of $2.6 million, with corresponding liabilities of $3.1 million on the 2019 consolidated balance sheets, including the reclassification of $0.5 million from deferred rent to right-of-use assets.

See Note 13 "Commitments and Contingencies" for lease policy note and additional disclosures.

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, 2019, our principal executive officer and principal financial and accountings officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level. The term "disclosure controls and procedures," as defined in Rules 13a-15(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 our financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, our principal executive and principal financial and accounting 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 U.S. GAAP. Our internal control over financial reporting includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect our transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with U.S. GAAP, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on our financial statements.

Our management, with the participation of our principal executive and principal financial and accounting officers, assessed the effectiveness of our internal control over financial reporting as of December 31, 2019. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in its 2013 *Internal Control — Integrated Framework*. Based on this assessment, our management has concluded that, as of December 31, 2019, our internal control over financial reporting is effective based on those criteria.

This Annual Report does not include an attestation report of our independent registered public accounting firm regarding internal control over financial reporting. Management's report was not subject to attestation by our independent registered public accounting firm pursuant to an exemption under Section 989G of the Dodd-Frank Wall Street Reform and Consumer Protection Act made available to us under the JOBS Act.

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.

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 2020 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

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 Index

Exhibit Number	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 (1)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (2)
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 (3)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (3)
3.4	Certificate of Amendment to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective June 25, 2018 (4)
3.5	Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of January 4, 2018 (5)
4.1	Form of Common Stock Certificate of Rocket Pharmaceuticals, Inc. (3)
4.2	Base Indenture, dated as of August 5, 2016, by and between Inotek Pharmaceuticals Corporation and Wilmington Trust, National Association (6)
4.3	First Supplemental Indenture, dated as of August 5, 2016, by and between Inotek Pharmaceuticals Corporation and Wilmington Trust, National Association (6)
4.4	Form of 5.75% Convertible Senior Note due 2021 (6)
4.5	Second Supplemental Indenture, dated as of February 20, 2020, by and between Rocket Pharmaceuticals, Inc. and Wilmington Trust, National Association (7)
4.6	Form 6.25% Convertible Senior Note due 2022 (7)
10.1#	2004 Stock Option and Incentive Plan (8)
10.2#	Rocket Pharmaceuticals, Inc. Second Amended and Restated 2014 Stock Option and Incentive Plan (9)
10.3#	Form of Incentive Stock Option Agreement (Employees) (10)
10.4#	Form of Non-Qualified Stock Option Agreement (Employees) (10)
10.5#	Form of Non-Qualified Stock Option Agreement (Non-Employee Directors) (10)
10.6#	Form of Non-Qualified Stock Option Agreement (Consultants) (10)
10.6.1#*	Form of Restricted Stock Unit Award Agreement
10.7#	Rocket Pharmaceuticals, Ltd. 2015 Share Option Plan (11)
10.8#	Letter Agreement, dated as of July 28, 2014, by and between the Registrant and David P. Southwell (8)
10.9#	Amendment to Offer Letter, effective as of September 1, 2017, by and between Inotek and David Southwell (12)
10.10#*	Offer Letter, dated September 25, 2019, by and between the registrant and Kamran Alam.
10.16#	Rocket Pharmaceuticals, Inc. Amended and Restated 2014 Employee Stock Purchase Plan (11)
10.17#	Form of Indemnification Agreement, to be entered into between the Registrant and its directors (3)
10.18#	Form of Indemnification Agreement, to be entered into between the Registrant and its officers (3)
10.19#	Form of Severance and Change of Control Agreements, to be entered into between the Registrant and certain of its officers (17)
10.20	Lease, dated as of May 29, 2015, by and between Inotek Pharmaceuticals Corporation and 91 Hartwell Avenue Trust, as amended and currently in effect (14)
10.21	First Amendment to Lease, dated as of February 24, 2016, by and between Inotek Pharmaceuticals Corporation and 91 Hartwell Avenue Trust (15)
10.22	Lease Agreement, dated as of March 31, 2016, by and between Rocket Pharmaceuticals, Ltd. and ARE-East River Science Park, LLC. (11)
10.23	Agreement of Lease, dated as of June 6, 2018, by and between Rocket Pharmaceuticals, Inc. and ESRT Empire State Building, L.L.C. (10)
10.24	Amendment No. 1 to the Lease Agreement, dated as of June 28, 2018, by and between Rocket Pharmaceuticals, Ltd. and ARE-East River Science Park, LLC (10)
10.26†	License Agreement, dated as of November 19, 2018, by and between Rocket Pharmaceuticals, Ltd. and REGENXBIO Inc. (16)
10.27	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 (2)
10.28	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 (2)

10.27**	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. (17)
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	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Link Document.

*	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.
(1)	Filed as an Exhibit 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.
(2)	Filed as an Exhibit 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)	Filed as an Exhibit 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)	Filed as an Exhibit 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.
(5)	Filed as an Exhibit to the Company's registration statement on Form 8-A, as amended (001-36829), filed with the SEC on January 11, 2018, and incorporated herein by reference.
(6)	Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on August 5, 2016, and incorporated herein by reference.
(7)	Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on February 20, 2020, and incorporated herein by reference.
(8)	Filed as an Exhibit 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.
(9)	Filed as an Exhibit to the Company's proxy statement on Schedule 14A (001-36829), filed with the SEC on April 30, 2018, as amended, and incorporated herein by reference.
(10)	Filed as an Exhibit to the Company's quarterly report on Form 10-Q (001-36829), filed with the SEC on August 14, 2018, as amended, and incorporated herein by reference.
(11)	Filed as an Exhibit 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.
(12)	Filed as an Exhibit 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.
(13)	Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on August 8, 2017, and incorporated herein by reference.
(14)	Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on June 1, 2015, and incorporated herein by reference.
(15)	Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on February 26, 2016, and incorporated herein by reference.
(16)	Filed as an Exhibit 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.
(17)	Filed as Exhibit 10.1 to the Company's quarterly report on Form 10-Q (001-36829), filed with the SEC on August 8, 2019, and incorporated herein by reference.

Item 16. Form 10-K Summary

None.

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 New York, State of New York, on March 6, 2020.

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 Kamran Alam, 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	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 6, 2020
/s/ Kamran Alam Kamran Alam	Senior Vice President - Finance *(Principal Financial Officer)*	March 6, 2020
/s/ John Militello John Militello	Senior Controller *(Principal Accounting Officer)*	March 6, 2020
/s/ Carsten Boess Carsten Boess	Director	March 6, 2020
/s/ Pedro Granadillo Pedro Granadillo	Director	March 6, 2020
/s/ Gotham Makker, MD Gotham Makker, MD	Director	March 6, 2020
/s/ David P. Southwell David P. Southwell	Director	March 6, 2020
/s/ Roderick Wong, MD Roderick Wong, MD	Director	March 6, 2020
/s/ Naveen Yalamanchi, MD Naveen Yalamanchi, MD	Director	March 6, 2020

Rocket Pharmaceuticals, Inc.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Rocket Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2019 and 2018, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity, and cash flows for each of the years the ended 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, 2019 and 2018, and the consolidated results of their operations and their cash flows for each of the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 13 to the consolidated financial statements, the Company has changed its method of accounting for leases for the year ended December 31, 2019, due to the adoption of Accounting Standard Update 2016-02, *Leases.*

Basis for Opinion

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

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

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

/s/ EisnerAmper LLP

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

EISNERAMPER LLP
New York, New York
March 6, 2020

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

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 185,383	$ 111,355
Investments	118,732	94,375
Prepaid expenses and other assets	3,639	3,358
Total current assets	307,754	209,088
Property and equipment, net	29,295	2,027
Goodwill	30,815	30,815
Internal use software	226	-
Restricted cash	1,525	1,436
Deposits	455	545
Operating lease right-of-use assets	2,051	-
Investments	-	7,402
Total assets	$ 372,121	$ 251,313
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 17,352	$ 15,372
Operating lease liabilities, current	957	-
Total current liabilities	18,309	15,372
Convertible notes, net of unamortized discount	45,049	41,447
Operating lease liabilities, non-current	1,443	-
Other liabilities	23	457
Total liabilities	64,824	57,276
Commitments and contingencies (Note 13)		
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 shares; 300,000 shares designated as Series B; 0 shares issued and outstanding	-	-
Common stock, $0.01 par value, 120,000,000 shares authorized; 54,773,061 and 45,194,736 shares issued and 54,769,030 and 45,144,736 shares outstanding at December 31, 2019 and 2018, respectively	548	452
Treasury stock, at cost, 4,031 and 50,000 common shares at December 31, 2019 and 2018, respectively	(53)	(668)
Additional paid-in capital	489,925	300,253
Accumulated other comprehensive income (loss)	20	(127)
Accumulated deficit	(183,143)	(105,873)
Total stockholders' equity	307,297	194,037
Total liabilities and stockholders' equity	$ 372,121	$ 251,313

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, 2019	2018
Revenue	$ -	$ -
Operating expenses:		
Research and development	58,623	53,270
General and administrative	17,528	17,886
Total operating expenses	76,151	71,156
Loss from operations	(76,151)	(71,156)
Research and development incentives	250	186
Interest expense	(5,958)	(6,039)
Interest and other income net	3,414	1,690
Accretion of discount on investments	1,175	801
Net loss	$ (77,270)	$ (74,518)
Net loss per share attributable to common stockholders - basic and diluted	$ (1.58)	$ (1.89)
Weighted-average common shares outstanding - basic and diluted	49,010,358	39,377,666

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, 2019	2018
Net loss	$ (77,270)	$ (74,518)
Other comprehensive loss		
Net unrealized gain (loss) on investments	147	(127)
Total comprehensive loss	$ (77,123)	$ (74,645)

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)

	Series A Convertible Preferred Shares		Series B Convertible Preferred Shares		Common Stock		Treasury Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount					
Balance at December 31, 2017	128,738	$ 16,060	126,909	$ 25,406	6,795,627	$ 68	$ -	$ 5,340	$ -	$ (31,355)	$ 15,519
Conversion of convertible preferred shares into common shares	(128,738)	(16,060)	(126,909)	(25,406)	19,475,788	194	-	41,272	-	-	-
Exchange of common shares in connection with the Reverse Merger	-	-	-	-	6,805,608	68	-	85,992	-	-	86,060
Issuance of common stock, net of issuance costs of $9.3 million	-	-	-	-	11,475,242	115	-	153,907	-	-	154,022
Issuance of common stock pursuant to settlement of restricted stock units	-	-	-	-	271,718	3	-	(3)	-	-	-
Issuance of common stock pursuant to exercise of stock options	-	-	-	-	370,753	4	-	144	-	-	148
Stock repurchase	-	-	-	-	-	-	(668)	-	-	-	(668)
Unrealized loss on investments	-	-	-	-	-	-	-	-	(127)	-	(127)
Stock-based compensation	-	-	-	-	-	-	-	13,601	-	-	13,601
Net loss	-	-	-	-	-	-	-	-	-	(74,518)	(74,518)
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
Stock-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

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,	
	2019	2018
Operating Activities:		
Net loss	$ (77,270)	$ (74,518)
Adjustments to reconcile net loss to net cash used in operating activities:		
Accretion of discount on convertible notes	3,602	3,059
Increase in lease liability	-	(161)
Depreciation expense	426	330
Stock-based compensation	13,371	13,601
Loss on disposal of property and equipment	-	317
Accretion of discount on investments	(1,066)	(801)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(917)	(1,505)
Accounts payable and accrued expenses	(2,722)	5,890
Operating lease liabilities	(87)	-
Net cash used in operating activities	(64,663)	(53,788)
Investing activities:		
Cash acquired in connection with the Reverse Merger	-	76,348
Purchases of investments	(184,298)	(141,087)
Proceeds from maturities of investments	168,556	61,276
Proceeds from sale of property and equipment	-	20
Purchases of property and equipment	(23,269)	(1,453)
Payment of security deposit	-	(376)
Net cash used in investing activities	(39,011)	(5,272)
Financing activities:		
Proceeds from issuance of common stock, net of issuance costs	177,760	154,022
Issuance of common stock, pursuant to exercise of stock options	31	148
Treasury stock repurchase	-	(668)
Proceeds from sale of treasury stock	344	-
Payment of witholding tax on option exercise	(344)	-
Net cash provided by financing activities	177,791	153,502
Net change in cash, cash equivalents and restricted cash	74,117	94,442
Cash, cash equivalents and restricted cash at beginning of year	112,791	18,349
Cash, cash equivalents and restricted cash at end of year	$ 186,908	$ 112,791
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment	$ 4,424	$ -
Accrued purchases of internal use software	$ 226	$ -
Treasury stock purchases paid in prior year	$ 726	$ -
Retirement of treasury stock	$ 1,393	$ -
Witholding tax payable on shares witheld in treasury stock	$ 53	$ -
Conversion of convertible preferred stock into common stock	$ -	$ 41,466
Unrealized gain (loss) on investments	$ 147	$ (127)
Supplemental cash flow information:		
Cash paid for interest	$ 2,990	$ 4,485
Cash paid for income taxes	$ 26	$ 2

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

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 pediatric diseases. We currently have three clinical-stage *ex vivo* lentiviral vector ("LVV") programs currently enrolling patients in the US and EU 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 US and EU. In addition, in the US 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. Finally, we have a pre-clinical stage LVV program for Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption – this program is anticipated to enter the clinic in 2020. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements. Additional work in the discovery stage for an FA CRISPR/CAS9 program as well as a gene therapy program for the less common FA subtypes C and G is ongoing.

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 $183.1 million as of December 31, 2019. As of December 31, 2019, the Company has $304.1 million of cash, cash equivalents and investments. During the year ended December 31, 2019, the Company received net proceeds of $177.8 million from the completion on April 18, 2019, and December 10, 2019 of public offerings of 5,175,000 and 4,393,000 shares of common stock, respectively. Rocket expects such resources will be sufficient to fund its operating expenses and capital expenditure requirements into 2022.

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 fair value under acquisition accounting, goodwill impairment, the accrual of research and development expenses, the valuation of equity transactions, lease liabilities and stock-based awards. Changes in estimates and assumptions are reflected in reported results in the period in which they become known. Actual results could differ from those estimates.

Cash, Cash Equivalents and Restricted Cash

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

Restricted cash consists of deposits collateralizing letters of credit issued by a bank in connection with the Company's operating leases (see Note 13 "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, 2019	December 31, 2018
Cash and cash equivalents	$ 185,383	$ 111,355
Restricted cash	1,525	1,436
	$ 186,908	$ 112,791

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 year ended December 31, 2019, we offset $1.2 million of grant funds against research and development ("R&D") expenses (See Note 16 "CIRM Grant" for additional disclosure).

Concentrations of credit risk and off-balance sheet risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents and available-for-sale securities. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company's marketable securities consist of U.S. Treasury securities, and Corporate, Government and Municipal 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, Government and Municipal 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 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, 2019 and 2018. There was $0.1 million of net unrealized gain and $0.1 million of net unrealized losses on investments for the years ended December 31, 2019 and 2018, 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 reviews goodwill for impairment at the consolidated level.

When testing goodwill, the Company has the option to first assess qualitative factors for reporting units that carry goodwill. The qualitative assessment includes assessing the totality of relevant events and circumstances that affect the fair value or carrying value of the reporting unit. These events and circumstances include macroeconomic conditions, industry and competitive environment conditions, overall financial performance, reporting unit specific events and market considerations. The Company also considers recent valuations of the reporting unit, including the magnitude of the difference between the most recent fair value estimate and the carrying value, as well as both positive and adverse events and circumstances, and the extent to which each of the events and circumstances identified may affect the comparison of a reporting unit's fair value with its carrying value. If the qualitative assessment results in a conclusion that it is more likely than not that the fair value of a reporting unit exceeds the carrying value, then no further testing is performed for that reporting unit.

In January 2017, the FASB issued ASU No. 2017-04, "Intangibles- Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment", an amendment to simplify the subsequent quantitative measurement of goodwill by eliminating step two from the goodwill impairment test. We early adopted this guidance as of January 1, 2018. An entity will recognize an impairment charge for the amount by which the carrying amount of a reporting unit exceeds its fair value, not to exceed the total amount of goodwill allocated to the reporting unit. An entity still has the option to perform the qualitative test for a reporting unit to determine if the quantitative impairment test is necessary. 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 for the years ended December 31, 2019, and 2018.

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 and construction costs 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.

Internal Use Software

The Company capitalizes certain external costs incurred to acquire and develop internal use software, principally related to our enterprise resource planning ("ERP") systems. As of December 31, 2019, the ERP system has not been activated.

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. Following this review, there is no impairment of long-lived assets for the years ended December 31, 2019 and 2018.

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. The fair value of the 2021 Convertible Notes as of December 31, 2019 was approximately $54.5 million. The Company's assets and liabilities measured at fair value on a recurring basis include its short and long term investments.

Research and Development

R&D costs, which include salaries and staff costs, license costs, 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, 2019 and 2018 are denominated in Euros. Gains and losses on foreign currency transactions were not significant for the years ended December 31, 2019 and 2018.

Treasury Stock

The Company records treasury stock at cost.

Stock-Based Compensation

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 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.

Effective July 1, 2018, the Company adopted ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"), which expands the scope of Topic 718 to include stock-based payment awards to nonemployees. As a result, stock-based awards granted to consultants and non-employees are accounted for in the same manner as awards granted to employees and directors as described above. Prior to the adoption of ASU 2018-07, for stock-based awards granted to consultants and non-employees, the Company recognized compensation expense over the period during which services are rendered by such consultants and non-employees until completed. At the end of each financial reporting period prior to completion of the service, the estimated fair value of these awards was re-measured using the then-current fair value of our common stock and updated assumption inputs in the Black-Scholes option-pricing model.

NYC Biotechnology Tax Credit Program

New York City allows 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 is 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, 2019 and 2018, the Company recorded research and development incentive income of $0.3 million and $0.2 million, respectively.

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 carry-forwards. 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, 2019 and 2018.

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.

Recent Accounting Pronouncements

Other than the adoption of ASU 2016-02 (see Note 13), there were no other recent accounting standards that impacted the Company for the year ended December 31, 2019.

4. 2018 Reverse-Merger Acquisition Accounting

On January 4, 2018, Rome Merger Sub ("Merger Sub"), a wholly owned subsidiary of Inotek Pharmaceuticals Corporation ("Inotek"), completed its merger with and into Rocket Pharmaceuticals, Ltd. ("Rocket Ltd"), with Rocket Ltd surviving as a wholly owned subsidiary of Inotek. This transaction is referred to as the "Reverse Merger." The Reverse Merger was effected pursuant to an Agreement and Plan of Merger and Reorganization (the "Merger Agreement"), dated as of September 12, 2017, by and among Inotek, Rocket Ltd and Rome Merger Sub.

The Reverse Merger has been accounted for as a reverse acquisition under the acquisition method of accounting where Rocket Ltd is considered the accounting acquirer and Inotek is the acquired company for financial reporting purposes. Rocket Ltd was determined to be the accounting acquirer based on the terms of the Merger Agreement and other factors, such as relative voting rights and the composition of the combined company's board of directors and senior management. The pre-acquisition financial statements of Rocket Ltd became the historical financial statements of Rocket following completion of the Reverse Merger. The historical financial statements, outstanding shares and all other historical share information have been adjusted by multiplying the respective share amount by the Exchange Ratio as if the Exchange Ratio had been in effect for all periods presented.

The identifiable assets and liabilities of Inotek are allocated in the Company's consolidated financial statements at their fair values at the acquisition date, January 4, 2018. Goodwill is calculated as the excess value of consideration paid over the fair value of assets acquired and liabilities assumed.

The acquisition-date fair value of the consideration transferred is as follows:

Number of shares of the combined company owned by Inotek shareholders	6,805,608
Number of shares issuable in connection with fully vested RSUs of Inotek immediately prior to the Reverse Merger	271,718
Inotek common stock on the acquisition date	7,077,326
Price per share of Inotek common stock on acquisition date	$ 12.16
Total purchase price	$ 86,060

The following table summarizes the fair value purchase price allocation of the assets acquired and liabilities assumed at the date of acquisition which is subject to adjustment as the Company finalizes it valuation:

Cash and cash equivalents	$ 76,348
Short term investments	21,292
Prepaid expense and other assets	1,041
Property and equipment	256
Deposits	168
Goodwill	30,815
Accounts payable and accrued expenses	(4,961)
Convertible notes	(38,388)
Unfavorable lease liability	(511)
Net assets acquired	$ 86,060

The goodwill of $30.8 million represents the premium over the purchase price. Goodwill is mainly attributable to the value of cash and cash equivalents and short term investments acquired as of the acquisition date and access to capital markets. The allocation of the purchase price with the assistance of a third party valuation, is based on certain management assumptions (Level 3 inputs).

5. 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, 2019 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Money market mutual funds (included in cash and cash equivalents)	$ 72,114	$ -	$ -	$ 72,114
Cash	7,542	-	-	7,542
	79,656	-	-	79,656
United States Treasury securities	75,464	-	-	75,464
Government Bonds	-	8,000	-	8,000
Corporate Bonds	-	29,268	-	29,268
Municipal Bonds	-	6,000	-	6,000
Investments	75,464	43,268	-	118,732
	$ 155,120	$ 43,268	$ -	$ 198,388

	Fair Value Measurements as of December 31, 2018 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market mutual funds (included in cash and cash equivalents)	$ 30,552	$ -	$ -	$ 30,552
United States Treasury securities	101,777	-	-	101,777
Investments	101,777	-	-	101,777
	$ 132,329	$ -	$ -	$ 132,329

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 Government, Corporate and Municipal Bonds as Level 2 assets as these assets are not traded in an active market and have been valued through a third-party pricing service based on quoted prices for similar assets.

The fair value of the 2021 Convertible Notes as of December 31, 2019 was approximately $54.5 million.

6. Property and Equipment

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

	December 31, 2019	December 31, 2018
Laboratory equipment	$ 1,910	$ 1,556
Computer equipment	179	179
Furniture and fixtures	273	273
Leasehold improvements	29	29
Construction costs in progress	27,809	469
	30,200	2,506
Less: accumulated depreciation	(905)	(479)
	$ 29,295	$ 2,027

Construction costs in progress comprise costs associated with the build out of the Company's research, manufacturing and office facilities under an operating lease in Cranbury, NJ. The costs are deemed to be landlord improvement costs. See Note 13 "Commitments and Contingencies" for additional disclosures. During the years ended December 31, 2019 and 2018, the Company capitalized interest in relation to the construction costs of $0.7 million and $0, and recognized $0.4 million and $0.3 million of depreciation expense, respectively.

7. Accounts Payable and Accrued Expenses

At December 31, 2019 and 2018, the Company's accounts payable and accrued expenses consisted of the following:

	December 31, 2019	December 31, 2018
Research and development	$ 7,418	$ 10,414
Construction costs in progress	4,424	-
Bonus	2,459	1,774
Accrued interest	1,241	1,241
Government grant payable	562	534
Professional fees	553	690
Accrued vacation	129	123
Severance and benefits	-	7
Internal use software	226	-
Other	340	589
	$ 17,352	$ 15,372

8. 2021 Convertible Notes Payable

On January 4, 2018, in connection with the Reverse Merger, Inotek's obligations under its outstanding convertible notes, with an aggregate principal value of $52.0 million, were assumed by the Company (the "2021 Convertible Notes"). The 2021 Convertible Notes were issued in 2016 and mature on August 1, 2021 ("Maturity Date"). The 2021 Convertible Notes are unsecured, accrue interest at a rate of 5.75% per annum and interest is payable semi-annually on February 1 and August 1 of each year. Each holder of a 2021 Convertible Notes (the "Holder") has 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 principal amount of 2021 Convertible Notes (the "Conversion Rate") which is $32.08 per share. The Conversion Rate is subject to adjustment from time to time upon the occurrence of certain events, including the issuance of stock dividends and payment of cash dividends.

In addition, in certain circumstances, the Conversion Rate will be increased in respect of a Holder's conversion of 2021 Convertible Notes in connection with the occurrence of one or more corporate events specified in the indenture (as supplemented, the "Indenture") governing the 2021 Convertible Notes (each such specified corporate event, a "Make-Whole Fundamental Change") that occurs prior to the Maturity Date (a "Make-Whole Fundamental Change Conversion") or in respect of a Holder's voluntary conversion of 2021 Convertible Notes other than in connection with a Make-Whole Fundamental Change (a "Voluntary Conversion"). In connection with a Make-Whole Fundamental Change Conversion or a Voluntary Conversion, the Company will increase the Conversion Rate for the 2021 Convertible Notes surrendered for conversion by a number of additional shares of the Company's common stock set forth in the Additional Shares Make-Whole Table in the Indenture, based on the applicable Stock Price (as defined in the Indenture) and Effective Date (as defined in the Indenture) for such conversion. The additional shares potentially issuable in connection with a Make-Whole Fundamental Change Conversion or a Voluntary Conversion range from 0 to 6.2375 per $1 principal amount of 2021 Convertible Notes, subject to adjustment. If the Stock Price applicable to any conversion is greater than $160.00 per share, the Conversion Rate will not be increased. If the Stock Price applicable to any conversion is less than $26.72 per share, the Conversion Rate in connection with a Make-Whole Fundamental Change Conversion will not be increased but it will be increased by 6.2375 shares in connection with a Voluntary Conversion. Upon conversion, Holders of the 2021 Convertible Notes will receive shares of the Company's common stock and cash in lieu of fractional shares.

Upon the occurrence of a Fundamental Change, the occurrence of certain change of control transactions or delisting events (as defined in the Indenture), each Holder may require the Company to repurchase for cash all or any portion of the 2021 Convertible Notes held by such Holder at a repurchase price equal to 100% of the principal amount thereof, plus accrued and unpaid interest thereon.

The Company, at its option, may redeem for cash all or any portion of the 2021 Convertible Notes if the last reported sale price of a share of the Company's common stock is equal to or greater than 200% of the conversion price for the 2021 Convertible Notes then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending within the five trading days immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2021 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date.

If an Event of Default (as defined in the Indenture), other than certain events of bankruptcy, insolvency or reorganization involving the Company, occurs and is continuing, the trustee under the Indenture or the Holders of at least 25% in principal amount of the outstanding 2021 Convertible Notes may declare 100% of the principal of and accrued and unpaid interest, if any, on all of the 2021 Convertible Notes to be due and payable immediately. Upon the occurrence of an Event of Default relating to bankruptcy, insolvency or reorganization involving the Company, 100% of the principal of and accrued and unpaid interest, if any, on all of the 2021 Convertible Notes would become due and payable automatically.

Notwithstanding the foregoing, the Indenture provides that, to the extent the Company elects, the sole remedy for an Event of Default relating to certain failures by the Company to comply with certain reporting covenants in the Indenture, will (i) for the first 90 days after the occurrence of such an Event of Default, consist exclusively of the right to receive additional interest on the 2021 Convertible Notes at a rate equal to 0.25% per annum of the principal amount of the 2021 Convertible Notes outstanding for each day during such 90-day period on which such an Event of Default is continuing and (ii) for the period from, and including, the 91st day after the occurrence of such an Event of Default to, and including, the 180th day after the occurrence of such an Event of Default, consist exclusively of the right to receive additional interest on the 2021 Convertible Notes at a rate equal to 0.50% per annum of the principal amount of the 2021 Convertible Notes outstanding for each day during such additional 90-day period on which such an Event of Default is continuing (such additional interest, "Additional Interest"). After 180 days, if such Event of Default is not cured or waived, the 2021 Convertible Notes would be subject to acceleration in accordance with the Indenture.

The 2021 Convertible Notes are considered a hybrid financial instrument consisting of a fixed interest rate "host" and various embedded features that required evaluation as potential embedded derivatives under FASB ASC 815, *Derivatives and Hedging* ("ASC 815"). Based on the nature of the host instrument and the embedded features, management concluded that none of the conversion, put and redemption features required bifurcation and separate accounting from the host instrument. The Company determined that the Additional Interest was an embedded derivative that contains non-credit related events of default. As a result, the Additional Interest feature required bifurcation and separate accounting under ASC 815. Based on the amount of Additional Interest that would be owed and the likelihood of occurrence, Rocket estimated that the fair value of the Additional Interest feature to be immaterial as of December 31, 2019 and 2018.

As of December 31, 2019, the stated interest rate was 5.75%, and the effective interest rate was 15.3%.

The table below summarizes the carrying value of the 2021 Convertible Notes:

	December 31, 2019	December 31, 2018
Principal amount	$ 52,000	$ 52,000
Discount	(6,951)	(10,553)
Carrying value	$ 45,049	$ 41,447

Accretion of the 2021 Convertible Notes discount was $3.6 million and $3.1 million for the years ended December 31, 2019 and 2018, respectively.

On February 10, 2020, the Company entered into separate, privately negotiated exchange agreements (the “Exchange Agreements”) with certain holders of the 2021 Convertible Notes (see Note 19).

9. Stockholders’ Equity

Treasury Stock

On December 27 and 28, 2018, the Company repurchased 100,000 shares of its common stock for aggregate consideration of approximately $1.4 million. The repurchases were made on the Nasdaq Stock Market at prevailing market prices in accordance with SEC Rule 10b-18. 50,000 of the shares repurchased at an average price of $13.36 by the Company settled on December 31, 2018 and was recorded as treasury stock. The remaining 50,000 shares repurchased at an average price of $14.50 resulting in $0.7 million, was recorded as a prepaid expense as of December 31, 2018. On January 2, 2019, these shares were settled and the $0.7 million was recorded as treasury stock. In January 2019, the total of 100,000 of treasury stock shares were retired and the total of $1.3 million was reclassified to additional paid in capital.

The Company sold 100,000 shares of common stock (the “Shares”) for $1.4 million to RTW Innovation Master Fund, Ltd. (“RTW Innovation”), which is an affiliate of the Company and is managed by RTW. RTW Innovation purchased the shares for a per-Share price of $13.93, plus an amount equal to the Company’s expenses incurred in connection with the Repurchases (including broker’s commissions) and the issuance of the Shares. The offering and sale of the Shares was completed as a private placement under Section 4(a) (2) of the Securities Act of 1933, as amended and included in the common shares outstanding as of December 31, 2018.

On June 25, 2019, the Company recorded treasury stock of $0.3 million for shares withheld to pay the payroll tax liability of an option exercise. These shares were sold in November 2019 for $0.3 million.

As of December 31, 2019, the Company recorded treasury stock of $0.1 million for the shares withheld to pay the payroll tax liability of an option exercise. The related payroll tax liability is included in the consolidated balance sheets as of December 31, 2019 within accounts payable and accrued expenses.

Public Offerings

On January 26, 2018, the Company completed a public offering of 6,325,000 shares of common stock, which included the full exercise of the underwriters’ options to purchase 825,000 additional shares of its common stock at a public offering price of $13.25 per share. The gross proceeds to Rocket from the January 2018 public offering were approximately $83.8 million, net of $5.3 million of offering costs, commission and legal and other expenses for net proceeds from the offering of $78.5 million.

On November 30, 2018, the Company completed a public offering of 4,082,500 shares of its common stock, which includes the full exercise of the underwriters’ option to purchase 532,500 additional shares of its common stock, at a public offering price of $15.50 per share. The gross proceeds to Rocket from the November 2018 public offering were approximately $63.3 million, net of $4.0 million of offering costs, commissions, legal and other expenses for a net proceeds from the offering of $59.3 million.

In addition to the shares sold in the November 2018 public offering, on November 30, 2018 Rocket completed a concurrent sale of 967,742 shares of common stock at a price of $15.50 per share, for gross proceeds of $15.0 million, net of offering costs of $0.2 million for net proceeds of $14.8 million, in a private placement to RTW Investments, LP, (“RTW”) an existing stockholder of the Company and an affiliate of Roderick Wong, the chairman of Rocket’s board of directors. The sale of these shares was not registered under the Securities Act of 1933, as amended, and such shares are subject to customary resale restrictions. Additionally, RTW signed a 90-day lock-up with respect to all shares of Rocket beneficially held by RTW.

On April 18, 2019, the Company completed a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters’ option to purchase an additional 675,000 shares of our common stock, at a public offering price of $17.50 per share. The gross proceeds to Rocket from the April 2019 public offering were approximately $90.6 million, after deducting $4.5 million of offering costs, commissions, legal and other expenses for a net proceeds from the offering of $86.1 million.

On December 10, 2019, the Company completed a public offering of 4,393,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 573,000 shares of our common stock, at a public offering price of $22.25 per share. The gross proceeds to Rocket from the December 2019 public offering were approximately $99.7 million, net of $6.1 million of offering costs, commissions, legal and other expenses for a net proceeds from the offering of $91.7 million.

10. Stock-Based Awards

Stock Option Valuation

Effective July 1, 2018, the Company adopted ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07").

The table below for the years ended December 31, 2019 and 2018 is post adoption of ASU 2018-07. 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,	
	2019	2018
Risk-free interest rate	2.26%	2.64%
Expected term (in years)	5.81	5.85
Expected volatility	75.71%	87.70%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 14.47	$ 17.82
Fair value of common stock	$ 14.47	$ 17.98

The following table summarizes stock option activity for the years ended December 31, 2019 and 2018, 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, 2017	6,959,347	$ 1.06	8.17	$ 27,175
Assumed as part of merger with Inotek	523,456	2.01	6.78	
Granted	1,650,878	17.82	9.26	
Exercised	(370,753)	1.27	-	
Forfeited	(146,931)	3.02		
Outstanding as of December 31, 2018	8,615,997	$ 4.48	7.51	$ 94,474
Granted	1,706,116	14.47	9.29	
Exercised	(110,325)	2.31		1,429
Cancelled	(448,247)	10.83		
Outstanding as of December 31, 2019	9,763,541	$ 5.96	6.96	164,021
Options vested and exercisable as of December 31, 2019	7,307,751	$ 3.06	6.30	$ 143,996
Options unvested as of December 31, 2019	2,455,790	$ 14.61	8.91	

The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2019 and 2018 was $9.58 and $13.21, respectively.

The total fair value of options vested during the years ended December 31, 2019 and 2018 was $63.1 million and $91.3 million, respectively.

Stock-Based Compensation

	Year Ended December 31, 2019	2018
Research and development	$ 6,153	$ 7,375
General and administrative	7,218	6,226
Total	$ 13,371	$ 13,601

As of December 31, 2019, the Company had an aggregate of $19.3 million of unrecognized stock-based compensation expense, which is expected to be recognized over the weighted average period of 1.74 years.

11. Net Loss Per Share

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

	For the Years Ended December 31, 2019	2018
Numerator:		
Net loss attributable to common stockholders	$ (77,270)	$ (74,518)
Denominator:		
Weighted-average common shares outstanding - basic and diluted	49,010,358	39,377,666
Net loss per share attributable to common stockholders - basic and diluted	$ (1.58)	$ (1.89)

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, 2019	2018
Shares issuable upon conversion of the 2021 Convertible Notes	1,620,948	1,620,948
Warrants exercisable for common shares	14,102	14,102
Options to purchase common shares	9,763,541	8,615,997
	11,398,591	10,251,047

The Company has warrants outstanding as of December 31, 2019 and 2018, convertible into 14,102 of common shares at an exercise price of $24.82 per share which expire on June 28, 2023.

12. Income Taxes

No provision for federal or state income taxes was recorded during the years ended December 31, 2019 and 2018, 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, 2019	2018
U.S. federal tax at statutory rate	21.0%	21.0%
Change in state tax apportionment	(17.4%)	15.8%
Stock compensation	(0.7%)	0.0%
Section 382 limitation	0.0%	(38.7%)
Valuation allowance	(5.1%)	3.5%
Other	2.1%	(1.6%)
Effective tax rate	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:

	For the years ended December 31, 2019	2018
Deferred income tax assets (liabilities)		
Net operating losses ("NOL") and credit carryforwards	$ 20,988	$ 28,400
Capitalized research and development costs	27,652	17,098
Other	643	1,385
Stock-based compensation	3,896	4,563
Debt discount	(1,460)	(3,652)
Valuation allowance	(51,719)	(47,794)
Net deferred income tax asset	$ -	$ -

As of December 31, 2019, the Company had federal and state net operating loss carryforwards of approximately $62.4 million and $2.2 million, respectively, which begin to expire in 2025. Additionally, $28.3 million of the Federal NOLs can be carried forward indefinitely.

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, 2019 and 2018. 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, 2019 and 2018, the valuation allowance increased by $3.9 million and $46.6 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 $93.0 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 $34.1 million. Additionally, $4.9 million of Federal R&D Credits will expire unutilized. As a result, in 2019, 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 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 recorded uncertain tax positions as liabilities in accordance with ASC 740 and adjust 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, 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, 2019 and 2018, no accrued interest or penalties are included on the related tax liability line in the consolidated balance sheets.

13. Commitments and Contingencies

The Company adopted ASU 2016-02, *Leases* ("ASU 2016-02"), as amended, on January 1, 2019, which supersedes the prior leasing guidance and upon adoption, requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet for all leases with terms longer than 12 months. Leases are classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Upon the adoption of the guidance, operating leases were capitalized on the balance sheet at the present value of lease payments. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 was calculated using an estimate of the Company's collateralized borrowing rate for debt with a similar term.

In adopting ASU 2016-02, the Company elected the available package of practical expedients which allows the Company to not reassess previous accounting conclusions around whether arrangements are or contain leases, the classification of leases, and the treatment of initial direct costs. The Company also made an accounting policy election to utilize the short-term lease exemption, whereby leases with a term of 12 months or less will not follow the recognition and measurement requirements of the new standard. Upon adoption, the Company recognized total right-of-use assets of $2.6 million, with corresponding liabilities of $3.1 million on the 2019 consolidated balance sheets, including the reclassification of $0.5 million from deferred rent to right-of-use assets.

The Company determines if an arrangement is a lease at inception. Operating leases are included in our balance sheet as right-of-use assets from operating leases, current operating lease liabilities and long-term operating 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. 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). 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.

Operating Leases

Rocket has entered into 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 will be dedicated to AAV Current Good Manufacturing Practice (cGMP) manufacturing to support the Company's pipeline, which has not yet commenced. 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"). We began to pay rent on September 1, 2019, and the NJ Lease Agreement has a term of 15 years from this date, with an option to renew for two consecutive five-year renewal terms. The NJ Lease Agreement commencement had not yet occurred at December 31, 2019, since the construction of all landlord owned improvements had not been completed, therefore as of December 31, 2019, the right of use asset and lease liability have not been recorded for this lease. These construction costs in progress will be included as part of the right of use asset upon the lease commencement date.

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, 2019 and 2018. The Company entered into the lease prior to the building being available for use as the building construction was not complete.

The total restricted cash balance for the Company's operating leases at December 31, 2019 and 2018 was $1.0 million and $0.9 million, respectively.

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"). In connection with the NY Lease Agreement, the Company established an irrevocable standby letter of credit ("ARE LOC") with a bank. The ARE LOC, in which the landlord is the beneficiary, serves as the Company's security deposit on the lease. The Company has a certificate of deposit with a bank as collateral for the ARE LOC which is classified as restricted cash in the consolidated balance sheets. On June 28, 2018, the Company entered into Amendment No.1 to the NY Lease Agreement, whereby the landlord agreed to relieve the Company of its obligations under the lease for a portion of the leased space upon the takeover of the space by a replacement tenant. The Company agreed to extend the lease for the remaining laboratory space by one additional year until July 2022. The Company recorded an additional rent expense of $0.1 million related to the early exit from the lease during the year end December 31, 2018. A replacement tenant has executed a new lease for the office space and as of September 7, 2018, the Company was relieved of its obligations for rent and security deposit for the office space. In October 2018, the Company provided a new letter of credit for $0.08 million (the "New ARE LOC") relating to the remaining lab space and the existing ARE LOC of $0.2 million was released back to the Company. The New ARE LOC expires and is automatically renewed April 8 of each succeeding calendar year until October 29, 2020, unless written notice is provided no later than 90 days before the then existing expiration date. The Company continues to maintain laboratory space at the Alexandria Center for Life Science facility as its hub for research and development activities.

On June 7, 2018, the Company entered into a three year lease agreement for office space in the Empire State Building (the "ESB Lease Agreement"). In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit (the "Empire LOC") with a bank for $0.9 million which expires on June 30, 2020 and is renewed automatically for a one year period until its expiration date of July 31, 2021. The Empire LOC serves as the Company's security deposit on the lease in which the landlord is the beneficiary. The Company has a certificate of deposit of $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, 2019.

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 $0.3 million and $0.1 million for the years ended December 31, 2019 and 2018, respectively.

Rent expense was $0.9 million and $0.8 million for the years ended December 31, 2019 and 2018, respectively.

Lease cost	**December 31, 2019**
Operating lease cost	$ 1,003
Total lease cost	$ 1,003

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, 2019:

Maturity of lease liabilities	**December 31, 2019**
2020	1,103
2021	894
2022	572
2023	73
Total lease payments	$ 2,642
Less: interest	(242)
Total operating lease liabilities	$ 2,400

The following disclosure is provided for periods prior to adoption of ASU 2016-02. Future annual minimum lease payment commitments, including the NJ Lease Agreement, as of December 31, 2019 were as follows:

2020	2,742
2021	2,530
2022	2,253
2023	1,802
2024	1,783
Thereafter	20,361
Total	$ 31,471

Leases	**December 31, 2019**
Operating right-of-use assets	$ 2,051
Operating current lease liabilities	957
Operating noncurrent lease liabilities	1,443
Total operating lease liabilities	$ 2,400

Other information

Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows from operating leases	$ 1,088
Weighted-average remaining lease term - operating leases	2.6 years
Weighted-average discount rate - operating leases	7.77%

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.

14. Agreements Related to Intellectual Property

The Company, directly and through its subsidiary Rocket Pharmaceuticals, Ltd. 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.

Contract Research and Collaboration Agreement with Lund University and J. Richter

In August 2016, Rocket entered into a research and collaboration agreement with Lund University and Johan Richter, M.D., Ph.D. under which Dr. Richter grants to Rocket an exclusive, perpetual, sublicensable, worldwide license to certain intellectual property rights of Dr. Richter relating to lentiviral-mediated gene transfer to treat IMO. In exchange for the license, Rocket is 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) and sublicense revenue payments to Dr. Richter. Under the terms of the agreement, Lund University and Dr. Richter are obligated to perform contract research for Rocket regarding the use of lentiviral-mediated gene transfer to treat IMO. Intellectual property resulting from the contract research created by Dr. Richter is included in the license described above and also subject to an option for Rocket to purchase ownership of such rights. Intellectual property created by Lund University in conducting such research is non-exclusively licensed to Rocket for non-commercial use and also subject to an option for Rocket to purchase or license such intellectual property under commercially reasonable terms. Rocket is obligated to pay for the contract research according to an agreed budget in quarterly installments in advance.

As consideration for an option to acquire rights from Lund University on commercially reasonable terms and conditions, Rocket paid Lund University an upfront license fee of €0.02 million (approximately $0.02 million), which was expensed as research and development costs. Rocket is obligated to make aggregate milestone payments of up to €0.1 million (approximately $0.1 million) to Lund University and Dr. Richter upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the Lund University agreement, Rocket is obligated to pay a low single digit percentage royalty on net sales, subject to specified adjustments, by Rocket or its sublicensees or affiliates. In the event that Rocket enters into a sublicense agreement with a sublicensee, Rocket will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances.

The research agreement had an initial term of 24 months. In August 2018, the research and collaboration agreement was amended for an additional year expiring August 2019 and amended in August 2019, renewing for an additional year expiring August 2020.

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, we are 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.

15. Strategic Research Collaboration

On May 16, 2018, Rocket and the Stanford University School of Medicine ("Stanford University") entered into a strategic collaboration to support the advancement of FA and PKD gene therapy research. Under the terms of the collaboration agreement, Stanford University will serve as the lead clinical trial research center in the U.S. for the planned FA registrational trial and would also be the lead U.S. site for PKD clinical trials. The project will also separately evaluate the potential for non-myeloablative, non-genotoxic antibody-based conditioning regimens as a future development possibility that may be applied across bone marrow-derived disorders. In addition, Rocket agreed to support expansion of Stanford University's Laboratory for Cell and Gene Therapy ("LCGM") in efforts to further enhance the development of Rocket's internal pipeline. Rocket agreed to fund up to $3.5 million for the LCGM expansion upon which 40% or $1.4 million was due upon execution of the agreement and the balance is due upon the achievement of certain milestones. $1.4 million of the $3.5 million milestone payments was paid during the year ended December 31, 2019. In January 2019, the Company and Stanford signed a Clinical Trial Agreement for the treatment of FA. Upon the signing of the January 2019 Stanford Clinical Trial Agreement, the second milestone of $1.4 million for the LGCM became due and was expensed in January 2019, when the milestone was met. As of December 31, 2019, none of the remaining milestones were met with regard to the LCGM.

16. CIRM Grant

On April 30, 2019, the CIRM awarded Rocket 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 I/II 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. On July 1, 2019, we received the first grant from CIRM of $0.8 million and on October 15, 2019, we received the second grant of $0.4 million, based on eligible costs incurred under the grant. The CIRM grant reimbursements are accrued as an offset against R&D expenses as reimbursable expenses are incurred.

17. Related Party Transactions

During March 2018, the Company entered into a consulting agreement with a member of the Board of Directors for strategic and finance consulting services to be provided to the Company. The Company incurred expenses of $3,600 and $0.2 million in connection with this consulting agreement for the years ended December 31, 2019 and 2018, respectively.

During April 2018, the Company entered into a consulting agreement with a different member of the Board of Directors for business development consulting services. The Company incurred expenses of $0.1 million in connection with this consulting agreement for both of the years ended December 31, 2019 and 2018.

18. 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, 2019 and 2018, was $0.2 million and $0.1 million, respectively.

19. Subsequent Events

Convertible Notes Exchange Agreement

On February 10, 2020, the Company entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of its outstanding 5.75% Convertible Senior Notes due 2021 (the "Old Notes") (see Note 8). Pursuant to the Exchange Agreements, we exchanged approximately $39.4 million aggregate principal amount of the Old Notes (which represents approximately 76% of the aggregate outstanding principal amount of the Old Notes) for (a) approximately $39.4 million aggregate principal amount of 6.25% Convertible Senior Notes due 2022 (the "New Notes") (an exchange ratio equal to 1.00 New Notes per exchanged Old Note) and (b) $119,416 in cash to pay the accrued and unpaid interest on the exchanged Old Notes from, and including, February 1, 2020, to, but excluding, the closing date of the exchange transactions. The New Notes were issued in private placements exempt from registration in reliance on Section 4(a) (2) of the Securities Act of 1933, as amended (the "Securities Act"). Upon completion of the exchange transactions, approximately $12.7 million aggregate principal amount of Old Notes remained outstanding. The exchange transactions closed on February 20, 2020.

The conversion rate for the New Notes will initially be 31.1876 shares of the Company's common stock per $1,000 principal amount of 2022 Notes, which is equivalent to an initial conversion price of approximately $32.06 per share of common stock, and is subject to adjustment under the terms of the New Notes. The Company may redeem for cash all or any portion of the 2022 Notes, at its option, if the last reported sale price of its common stock is equal to or greater than 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending within the five trading days immediately preceding the date on which the Company provides written notice of redemption. The 2022 Notes Indenture contains customary terms and covenants and events of default. We are currently reviewing the accounting for this transaction.

Additionally, the Company repurchased 3,000 shares of its common stock at an aggregate amount of $71,670 from certain holders of the Old Notes participating in the exchange transactions in privately negotiated, private transactions.

Exhibit 10.6.1

ROCKET PHARMACEUTICALS, INC.

SECOND AMENDED AND RESTATED 2014 STOCK OPTION AND INCENTIVE PLAN

RESTRICTED STOCK UNIT AWARD AGREEMENT

Unless otherwise defined herein, the terms defined in the Rocket Pharmaceuticals, Inc. Second Amended and Restated 2014 Stock Option and Incentive Plan (the "***Plan***") will have the same defined meanings in this Restricted Stock Unit Award Agreement (the ***"Award Agreement"***).

I. NOTICE OF RESTRICTED STOCK UNIT GRANT

Participant Name:

You have been granted the right to receive an Award of Restricted Stock Units, subject to the terms and conditions of the Plan and this Award Agreement, as follows:

Grant Number _____

Date of Grant _____

Vesting Commencement Date _____

Number of Restricted Stock Units _____

Vesting Schedule:

Subject to Section 3 of the Award Agreement, the Restricted Stock Units will vest in accordance with the following schedule:

[Insert Vesting Schedule]

Notwithstanding the foregoing or any provision of the Plan or the Award Agreement to the contrary, if a Sale Event (as defined in the Plan) occurs and the Participant's status as an Employee is terminated by the Company or a Subsidiary without Cause or by the Participant for Good Reason (as defined herein) within 12 months following the Sale Event, 100% of the Restricted Stock Units shall become immediately vested (an **"Acceleration of Vesting"**). "**Good Reason**" means the occurrence, without the Participant's express written consent, which circumstances are not remedied by the Company within thirty (30) days of its receipt of a written notice from the Participant describing the applicable circumstances (which notice must be provided by the Participant within ninety (90) days of the Participant's knowledge of the applicable circumstances), of one or more of the following: (a) any material, adverse change in the Participant's duties, responsibilities, authority, title or reporting structure; (b) a material reduction in the Participant's base salary or bonus opportunity; or (c) a geographical relocation of the Participant's principal office location by more than fifty (50) miles.

In the event Participant ceases to be an Employee for any or no reason before Participant vests in the Restricted Stock Unit, the Restricted Stock Unit and Participant's right to acquire any Stock hereunder will terminate in accordance with Section 3 of the Award Agreement.

By Participant's signature and the signature of the representative of Rocket Pharmaceuticals, Inc. (the ***"Company"***) below, or by Participant otherwise accepting this Award, Participant and the Company agree that this Award of Restricted Stock Units is granted under and governed by the terms and conditions of the Plan and this Award Agreement, including the Terms and Conditions of this Notice of Restricted Stock Unit Grant (including any country-specific addendum thereto), attached hereto as Exhibit A, all of which are made a part of this document. Participant has reviewed the Plan and this Award Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Award Agreement and fully understands all provisions of the Plan and Award Agreement. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and Award Agreement.

PARTICIPANT:	ROCKET PHARMACEUTICALS, INC.
Signature	By
Print Name	Title

EXHIBIT A

TERMS AND CONDITIONS OF RESTRICTED STOCK UNIT GRANT

1. Grant. The Company hereby grants to the individual named in the Notice of Restricted Stock Unit Grant attached as Part I of this Award Agreement (the "***Participant***") under the Plan an Award of Restricted Stock Units, subject to all of the terms and conditions in this Award Agreement and the Plan, which is incorporated herein by reference. Subject to Section 16 of the Plan, in the event of a conflict between the terms and conditions of the Plan and the terms and conditions of this Award Agreement, the terms and conditions of the Plan will prevail.

2. Company's Obligation to Pay. Each Restricted Stock Unit represents the right to receive Stock on the date it vests. Unless and until the Restricted Stock Units will have vested in the manner set forth in Section 3, Participant will have no right to payment of any such Restricted Stock Units. Prior to actual payment of any vested Restricted Stock Units, such Restricted Stock Units will represent an unsecured obligation of the Company, payable (if at all) only from the general assets of the Company. Any Restricted Stock Units that vest in accordance with Section 3 will be paid to Participant (or in the event of Participant's death, to his or her estate) in whole shares of Stock as set forth herein, subject to Participant satisfying any Tax-Related Items as set forth in Section 7. Subject to the provisions of Section 4, such vested Restricted Stock Units will be paid in whole shares of Stock as soon as practicable after vesting, but in each such case within the period ending no later than the date that is two and one-half (2½) months from the end of the Company's tax year that includes the vesting date. In no event will Participant be permitted, directly or indirectly, to specify the taxable year of the payment of any Restricted Stock Units payable under this Award Agreement.

3. Vesting Schedule. Subject to Section 5, the Restricted Stock Units awarded by this Award Agreement will vest in accordance with the vesting provisions set forth in the Notice of Restricted Stock Unit Grant. Restricted Stock Units scheduled to vest on a certain date or upon the occurrence of a certain condition will not vest in accordance with any of the provisions of this Award Agreement, unless Participant will have been continuously an employee of the Company, or any parent or Subsidiary of the Company (an **"Employee"**) from the date of grant until the date such vesting occurs. Employee status for purposes of this Award will end on the day that Participant is no longer actively providing services as an Employee and will not be extended by any notice period or "garden leave" that may be required contractually or under applicable laws. Notwithstanding the foregoing, the Administrator (or any delegate) shall have the sole and absolute discretion to determine when Participant is no longer providing active service for purposes of Employee status and participation in the Plan.

4. Administrator Discretion. Notwithstanding anything in the Plan or this Award Agreement to the contrary, if the vesting of the balance, or some lesser portion of the balance, of the Restricted Stock Units is accelerated in connection with Participant's termination as an Employee (provided that such termination is a "separation from service" within the meaning of Section 409A, as determined by the Company), other than due to death**,** and if (x) Participant is a "specified employee" within the meaning of Section 409A at the time of such termination as an Employee and (y) the payment of such accelerated Restricted Stock Units will result in the imposition of additional tax under Section 409A if paid to Participant on or within the six (6) month period following Participant's termination as an Employee, then the payment of such accelerated Restricted Stock Units will not be made until the date six (6) months and one (1) day following the date of Participant's termination as a Employee, unless the Participant dies following his or her termination as an Employee, in which case, the Restricted Stock Units will be paid in Stock to the Participant's estate as soon as practicable following his or her death. It is the intent of this Award Agreement that it and all payments and benefits hereunder be exempt from, or comply with, the requirements of Section 409A so that none of the Restricted Stock Units provided under this Award Agreement or Stock issuable thereunder will be subject to the additional tax imposed under Section 409A, and any ambiguities herein will be interpreted to be so exempt or so comply. Each payment payable under this Award Agreement is intended to constitute a separate payment for purposes of U.S. Treasury Regulation Section 1.409A-2(b)(2). For purposes of this Award Agreement, "Section 409A" means Section 409A of the Code, and any final U.S. Treasury Regulations and U.S. Internal Revenue Service guidance thereunder, as each may be amended from time to time.

5. Forfeiture upon Termination of Status as an Employee. Notwithstanding any contrary provision of this Award Agreement and subject to any Acceleration of Vesting, the balance of the Restricted Stock Units that have not vested as of the time Participant's status as an Employee is terminated will cease vesting in accordance with Section 3 above and Participant's right to acquire any Stock hereunder will immediately terminate.

6. Death of Participant. To the extent permitted by the Company, Participant may designate a beneficiary or beneficiaries to receive any payment hereunder that is payable on or after the Participant's death. Any such designation shall be on a form provided for that purpose by the Administrator and shall not be effective until received by the Administrator. If no beneficiary has been designated by a deceased Participant, or if the designated beneficiaries have predeceased the Participant, or if the beneficiary designation is deemed ambiguous, incomplete or invalid by the Administrator, the beneficiary shall be the Participant's estate.

7. Withholding of Taxes. Regardless of any action the Company or Participant's employer (the ***"Employer"***) takes with respect to any or all applicable national, local, or other tax or social contribution, withholding, required deductions, or other payments, if any, that arise upon the grant or vesting of the Restricted Stock Units or the holding or subsequent sale of Stock, and the receipt of Dividend Equivalent Rights or dividends, if any, or otherwise in connection with the Restricted Stock Units or Stock (***"Tax-Related Items"***), Participant acknowledges and agrees that the ultimate liability for all Tax-Related Items legally due by Participant is and remains Participant's responsibility and may exceed any amount actually withheld by the Company or the Employer. Participant further acknowledges and agrees that Participant is solely responsible for filing all relevant documentation that may be required in relation to the Restricted Stock Units or any Tax-Related Items (other than filings or documentation that is the specific obligation of the Company or a parent, Subsidiary, or Employer pursuant to applicable law) such as but not limited to personal income tax returns or reporting statements in relation to the grant, vesting or settlement of the Restricted Stock Units, the holding of Stock or any bank or brokerage account, the subsequent sale of Stock, and the receipt of any Dividend Equivalent Rights or dividends, if any. Participant further acknowledges that the Company and the Employer (a) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of the Restricted Stock Units, including grant or vesting, the subsequent sale of Stock acquired under the Plan, and the receipt of Dividend Equivalent Rights or dividends, if any; and (b) does not commit to and is under no obligation to structure the terms of the Restricted Stock Units or any aspect of the Restricted Stock Units to reduce or eliminate Participant's liability for Tax- Related Items, or achieve any particular tax result. Participant also understands that applicable laws may require varying Stock or Restricted Stock Unit valuation methods for purposes of calculating Tax-Related Items, and the Company assumes no responsibility or liability in relation to any such valuation or for any calculation or reporting of income or Tax-Related Items that may be required of Participant under applicable laws. Further, if Participant has become subject to tax in more than one jurisdiction between the date of grant and the date of any relevant taxable event, Participant acknowledges that the Company and/or the Employer (or former employer, as applicable) may be required to withhold or account for Tax-Related Items in more than one jurisdiction. Notwithstanding any contrary provision of this Award Agreement, no certificate representing the Stock will be issued to Participant, unless and until satisfactory arrangements (as determined by the Administrator) will have been made by Participant with respect to the payment of any Tax-Related Items which the Company determines must be withheld with respect to such Stock.

As a condition to the grant and vesting of the Restricted Stock Units and as set forth in Section 14 of the Plan, Participant hereby agrees to make adequate provision for the satisfaction of (and will indemnify the Company and any parent or Subsidiary for) any Tax-Related Items. Subject to approval by the Administrator, the Tax-Related Items shall be satisfied by the Company's withholding all or a portion of any Stock that otherwise would be issued to Participant upon payment of the vested Restricted Stock Units; provided that amounts withheld shall not exceed the amount necessary to satisfy the Company's minimum tax withholding obligations. Such withheld Stock shall be valued based on the Fair Market Value as of the date the withholding obligations are satisfied. Furthermore, Participant agrees to pay the Company or any parent, Subsidiary, or Employer any Tax-Related Items that cannot be satisfied by the foregoing methods.

8. Rights as Stockholder. Neither Participant nor any person claiming under or through Participant will have any of the rights or privileges of a stockholder of the Company in respect of any Stock deliverable hereunder unless and until certificates representing such Stock will have been issued, recorded on the records of the Company or its transfer agents or registrars, and delivered to Participant; provided, however, that the Participant may be credited with Dividend Equivalent Rights with respect to the stock units underlying the Restricted Stock Units, subject to the provisions of Section 12 of the Plan and such terms and conditions as the Administrator may determine. After such issuance, recordation and delivery, Participant will have all the rights of a stockholder of the Company with respect to voting such Stock and receipt of dividends and distributions on such Stock, but prior to such issuance, Participant will not have any rights to dividends and/or distributions on such Stock.

9. No Guarantee of Continued Service or Grants. PARTICIPANT ACKNOWLEDGES AND AGREES THAT THE VESTING OF THE RESTRICTED STOCK UNITS PURSUANT TO THE VESTING SCHEDULE HEREOF SHALL OCCUR ONLY BY CONTINUING AS AN EMPLOYEE AT THE WILL OF THE EMPLOYER OR CONTRACTING ENTITY (AS APPLICABLE) AND NOT THROUGH THE ACT OF BEING HIRED, BEING GRANTED THIS AWARD OF RESTRICTED STOCK UNITS OR ACQUIRING STOCK HEREUNDER. PARTICIPANT FURTHER ACKNOWLEDGES AND AGREES THAT THIS AWARD AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREUNDER AND THE VESTING SCHEDULE SET FORTH HEREIN DO NOT CONSTITUTE AN EXPRESS OR IMPLIED PROMISE OF CONTINUED ENGAGEMENT AS AN EMPLOYEE FOR THE VESTING PERIOD, FOR ANY PERIOD, OR AT ALL, AND WILL NOT INTERFERE IN ANY WAY WITH PARTICIPANT'S RIGHT OR THE RIGHT OF THE COMPANY (OR ANY PARENT, SUBSIDIARY, OR EMPLOYER) TO TERMINATE PARTICIPANT'S RELATIONSHIP AS AN EMPLOYEE AT ANY TIME, WITH OR WITHOUT CAUSE, SUBJECT TO APPLICABLE LAWS.

Participant also acknowledges and agrees that: (a) the Plan is established voluntarily by the Company, it is discretionary in nature and it may be modified, amended, suspended or terminated by the Company at any time; (b) the grant of Restricted Stock Units is voluntary and occasional and does not create any contractual or other right to receive future grants of Restricted Stock Units, or benefits in lieu of Restricted Stock Units even if Restricted Stock Units have been granted repeatedly in the past; (c) all decisions with respect to future awards of Restricted Stock Units, if any, will be at the sole discretion of the Company; (d) Participant's participation in the Plan is voluntary; (e) the Restricted Stock Units and the Stock subject to the Restricted Stock Units are extraordinary items that do not constitute regular compensation for services rendered to the Company or the Employer, and that are outside the scope of Participant's employment contract, if any; (f) the Restricted Stock Units and the Stock subject to the Restricted Stock Units are not intended to replace any pension rights or compensation; (g) the Restricted Stock Units and the Stock subject to the Restricted Stock Units are not part of normal or expected compensation or salary for any purposes, including, but not limited to, calculating any severance, resignation, termination, redundancy, dismissal, or end of service payments, bonuses, long-service awards, pension or retirement or welfare benefits or similar payments and in no event should be considered as compensation for, or relating in any way to, past services for the Company or the Employer, subject to applicable laws.

10. Address for Notices. Notices hereunder shall be mailed or delivered to the Company at its principal place of business and shall be mailed or delivered to the Participant at the address on file with the Company or, in either case, at such other address as one party may subsequently furnish to the other party in writing.

11. Grant is Not Transferable. Except to the limited extent provided in Section 6 above or as otherwise provided in Section 13 of the Plan, no Awards of Restricted Stock Units shall be sold, assigned, transferred or otherwise encumbered or disposed of by a Participant other than by will or by the laws of descent and distribution or pursuant to a domestic relations order. No Awards of Restricted Stock Units shall be subject, in whole or in part, to attachment, execution, or levy of any kind, and any purported transfer in violation hereof shall be null and void.

12. Binding Agreement. Subject to the limitation on the transferability of this grant contained herein, this Award Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

13. Additional Conditions to Issuance of Stock and Imposition of Other Requirements. If at any time the Company determines, in its discretion, that the listing, registration, qualification or rule compliance of the Stock upon any securities exchange or under any applicable laws, the Code or the consent or approval of any governmental regulatory authority is necessary or desirable as a condition to the issuance of Stock to Participant (or his or her estate) hereunder, such issuance will not occur unless and until such listing, registration, qualification, rule compliance, consent or approval will have been completed, effected or obtained free of any conditions not acceptable to the Company. Where the Company determines that the delivery of the payment of any Stock will violate any state, federal or foreign securities or exchange laws or other applicable laws, the Company will defer delivery until the earliest date at which the Company reasonably anticipates that the delivery of Stock will no longer cause such violation. The Company will make all reasonable efforts to meet the requirements of any applicable laws or securities exchange and to obtain any such consent or approval of any such governmental authority or securities exchange. The Company shall not be obligated to issue any Stock pursuant to the Restricted Stock Units at any time if the issuance of Stock violates or is not in compliance with any applicable laws, rules or regulations of the United States or any state or country.

Furthermore, the Company reserves the right to impose other requirements on Participant's participation in the Plan, on the Restricted Stock Units and on any Stock acquired under the Plan, to the extent the Company determines it is necessary or advisable in order to comply with any applicable laws or facilitate the administration of the Plan, and to require Participant to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing. Furthermore, Participant understands that the applicable laws of the country in which he or she is resident at the time of grant or vesting of the Restricted Stock Units or the holding or disposition of Stock (including any rules or regulations governing securities, foreign exchange, tax, labor or other matters) may restrict or prevent the issuance of Stock or may subject Participant to additional procedural or regulatory requirements he or she is solely responsible for and will have to independently fulfill in relation to the Restricted Stock Units or the Stock. Notwithstanding any provision herein, the Restricted Stock Units and any Stock shall be subject to any special terms and conditions or disclosures as set forth in any addendum for Participant's country (the "Country- Specific Addendum," which forms part of this Award Agreement). Participant also understands and agrees that if he works, resides, moves to, or otherwise is or becomes subject to applicable laws or company policies of another jurisdiction at any time, certain country-specific notices, disclaimers and/or terms and conditions may apply to him as from the date of grant, unless otherwise determined by the Company in its sole discretion.

14. Plan Governs. This Award Agreement is subject to all terms and provisions of the Plan. In the event of a conflict between one or more provisions of this Award Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Award Agreement will have the meaning set forth in the Plan.

15. Administrator Authority. As provided more fully in Section 2(b) of the Plan, the Administrator will have the power to interpret the Plan and this Award Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret or revoke any such rules (including, but not limited to, the determination of whether or not any Restricted Stock Units have vested). All actions taken and all interpretations and determinations made by the Administrator in good faith will be final and binding upon Participant, the Company and all other interested persons. No member of the Administrator will be personally liable for any action, determination or interpretation made in good faith with respect to the Plan or this Award Agreement.

16. Electronic Delivery and Acceptance; Translation. The Company may, in its sole discretion, decide to deliver any documents related to Restricted Stock Units awarded under the Plan or future Restricted Stock Units that may be awarded under the Plan by electronic means or request Participant's consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company. If Participant has received this Award Agreement, including appendices, or any other document related to the Plan translated into a language other than English, and the meaning of the translated version is different than the English version, the English version will control.

17. Captions. Captions provided herein are for convenience only and are not to serve as a basis for interpretation or construction of this Award Agreement.

18. Agreement Severable. In the event that any provision in this Award Agreement will be held invalid or unenforceable, such provision will be severable from, and such invalidity or unenforceability will not be construed to have any effect on, the remaining provisions of this Award Agreement.

19. Modifications to the Award Agreement. This Award Agreement constitutes the entire understanding of the parties on the subjects covered. Participant expressly warrants that he or she is not accepting this Award Agreement in reliance on any promises, representations, or inducements other than those contained herein. Modifications to this Award Agreement or the Plan can be made only in an express written contract executed by a duly authorized officer of the Company. Notwithstanding anything to the contrary in the Plan or this Award Agreement, the Company reserves the right to revise this Award Agreement as it deems necessary or advisable, in its sole discretion and without the consent of Participant, to comply with Section 409A or to otherwise avoid imposition of any additional tax or income recognition under Section 409A in connection to this Award of Restricted Stock Units.

20. Data Privacy. ***Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of Participant's Personal Data (as described below) by and among, as applicable, the Company, any parent, Subsidiary, or affiliate, or third parties as may be selected by the Company for the exclusive purpose of implementing, administering and managing Participant's participation in the Plan. Participant understands that refusal or withdrawal of consent will affect Participant's ability to participate in the Plan; without providing consent, Participant will not be able to participate in the Plan or realize benefits (if any) from the Restricted Stock Unit.***

Participant understands that the Company and any parent, Subsidiary, affiliate, or designated third parties may hold personal information about Participant, including, but not limited to, Participant's name, home address and telephone number, date of birth, social insurance number or other identification number, salary, nationality, job title, any shares of stock or directorships held in the Company or any parent, Subsidiary, or affiliate, details of all Restricted Stock Units or any other entitlement to Stock awarded, canceled, exercised, vested, unvested or outstanding in Participant's favor ("Personal Data"). Participant understands that Personal Data may be transferred to any parent, Subsidiary, affiliate, or third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in the United States, Participant's country (if different than the United States), or elsewhere, and that the recipient's country may have different data privacy laws and protections than Participant's country. In particular, the Company may transfer Personal Data to the broker or stock plan administrator assisting with the Plan, to its legal counsel and tax/accounting advisor, and to the affiliate or entity that is Participant's employer and its payroll provider.

Participant should also refer to any data privacy policy implemented by the Company (which will be available to Participant separately and may be updated from time to time) for more information regarding the collection, use, storage, and transfer of Participant's Personal Data.

21. Foreign Exchange Fluctuations and Restrictions. Participant understands and agrees that the future value of the underlying Stock is unknown and cannot be predicted with certainty and may decrease. Participant also understands that neither the Company, nor any affiliate is responsible for any foreign exchange fluctuation between local currency and the United States Dollar or the selection by the Company or any affiliate in its sole discretion of an applicable foreign currency exchange rate that may affect the value of the Restricted Stock Units or Stock received (or the calculation of income or Tax-Related Items thereunder). Participant understands and agrees that any cross-border remittance made to transfer proceeds received upon the sale of Stock must be made through a locally authorized financial institution or registered foreign exchange agency and may require the Participant to provide such entity with certain information regarding the transaction.

22. Amendment, Suspension or Termination of the Plan. By accepting this Award, Participant expressly warrants that he or she has received an Award of Restricted Stock Units under the Plan, and has received, read and understood a description of the Plan. Participant understands that the Plan is discretionary in nature and may be amended, suspended or terminated by the Company at any time.

23. Governing Law. This Award Agreement will be governed by the laws of the State of Delaware, without giving effect to the conflict of law principles thereof.

***

ATTACHMENT A

COUNTRY-SPECIFC ADDENDUM
(See Attached)

Exhibit 10.10

rocket

350 Fifth Avenue, Suite 7530
New York, NY 10118

430 East 29th Street, Suite 1040
New York, NY10016

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

September 25, 2019

Dear Kamran,

Rocket Pharmaceuticals, Inc. (the "Company") is pleased to offer you the following terms of employment as Senior Vice President, Finance, on a date to be agreed upon in 2019 "Start Date", reporting to the CEO & President. The initial terms of your new position with the Company are as set forth below.

1. Position.

You will be a Senior Vice President, Finance for the Company. Your responsibilities in this position will be agreed upon with the CEO prior to start of employment and formalized through the objective setting process under the existing Rocket performance management process within the first two weeks of start.

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.

2. Compensation.

Your base salary will be at the rate of $300,000 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 35% 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 Company in its sole and absolute discretion. As your start date will be in the 4th quarter of 2019 and normally you would not be eligible for a 2019 bonus, the expected bonus payout from your prior employer has been taken into account for the sign-on bonus (see below).

You will receive 17 days of vacation annually. Your vacation will be prorated for 2019 based on your Start Date. Beyond 2019, your vacation will vest according to company policy with a starting base of 17 days being in effect and up to 5 days of vacation carry over to the next year.

You will also receive a sign-on/relocation bonus of $130,000 (the "sign-on bonus"), with $80,000 payable on the Company's next regularly scheduled pay date following your Start Date, and $50,000 payable on or before March 15 timed with regular Rocket bonus payouts. If you leave the Company prior to the one year anniversary of your Start Date, you are responsible for reimbursement of the sign-on bonus to the Company within 30 days following the date of termination of employment.

In addition, you will 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 401(k) plan with 4% Company match and immediate 100% vesting.

3. Stock Options Grant.

Subject to applicable approval by the Board of Directors or a committee thereof, you will be issued stock options to purchase 200,000 of the Company's common ordinary shares ("the option") pursuant to the applicable share option plan through which the shares will be issued. The grant date of the option shall be the next regularly scheduled monthly Company grant date (the "Grant Date") following your Start Date. The option shall vest as follows: 1/3 shall vest upon the one-year anniversary of your Grant Date and the remaining 2/3 shall vest over the subsequent two years, with one-eighth (1/8) of the remaining options vesting each quarter following the one-year anniversary of your Grant Date. The option vesting schedule and the options shall be subject to the terms of your option agreement, including the applicable expiration period.

4. At-Will Employment & Termination.

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.

5. Compliance with Legal Obligations

By accepting this offer, you confirm that you are able to accept and perform this job without breaching any legal restrictions on your activities, such as restrictions imposed by a current or former employer. You also confirm that you will inform the Company about any such restrictions and provide the Company with as much information about them as possible, including any agreements between you and your current or former employer describing such restrictions on your activities. You further confirm that you will not remove or take any documents or proprietary data or materials of any kind, electronic or otherwise, with you from your current or former employer to the Company without written authorization from your current or former employer, nor will you use or disclose any such confidential information during the course and scope of your employment with the Company. If you have any questions about the ownership of particular documents or other information, discuss such questions with your former employer before removing or copying the documents or information.

6. Additional Information

This letter contains all of the terms of your employment with the Company and supersedes 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, satisfactory results of a drug test and/or background test.

If you have any questions about the above details, please call me at your earliest convenience. If you wish to accept this position, please sign below and return to me at your earliest convenience.

We look forward to welcoming you to Rocket!

Sincerely,

/s/ Gaurav D. Shah Date: 9.27.19

Gaurav D. Shah, MD

President & CEO, Rocket Pharmaceuticals, Inc.

Agreed and accepted:

/s/ Kamran Alam Date: 9/26/2019

Kamran Alam

Exhibit 21.1

Subsidiaries of Rocket Pharmaceuticals, Inc.

	Subsidiary	Jurisdiction of Incorporation	Rocket Ownership
1.	Inotek Securities Corporation	Massachusetts	100%
2.	Rocket Pharmaceuticals, Ltd.	Cayman Islands	100%
3.	Rocket Foundation, Inc.	Delaware	100%

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statements of Rocket Pharmaceuticals, Inc. on Form S-3 (No. 333-232168) and Forms S-8 (Nos. 333-204501, 333-212308, 333-216892 and 333-223488) of our report dated March 6, 2020, on our audits of the consolidated financial statements as of December 31, 2019 and 2018 and for each of the years then ended, which report is included in this Annual Report on Form 10-K to be filed on or about March 6, 2020.

/s/ EISNERAMPER LLP

EISNERAMPER LLP
New York, New York
March 6, 2020

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: March 6, 2020

/s/ Gaurav Shah, MD

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

Exhibit 31.2

Certifications under Section 302

I, Kamran Alam, 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 my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 6, 2020

/s/ Kamran Alam

Kamran Alam
Senior Vice President, Finance
(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, 2019, 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 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: March 6, 2020

/s/ Gaurav Shah, MD

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

Date: March 6, 2020

/s/ Kamran Alam

Kamran Alam
Senior Vice President, Finance (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.