

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, 2018
OR

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

For the transition period from _____ to _____

Commission File Number: 001-36829

Rocket Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction of Incorporation or Organization)

04-3475813
(IRS Employer Identification No.)

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 on its corporate Web site, 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 if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

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. (Check one):

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company	<input type="checkbox"/>
Emerging growth company	<input checked="" type="checkbox"/>		

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 voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2018 was approximately \$428.9 million, based upon the closing price on the Nasdaq Global Market reported for such date. Shares of the registrant's common stock held by each officer and director and by each person who is known to own 10% or more of the outstanding common stock have been excluded in that such persons may be deemed to be affiliates of the Company. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 1, 2019, there were 45,114,437 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 2019 Annual Meeting of Stockholders or an amendment to this Annual Report on Form 10-K, in any case, to be filed within 120 days of the end of the period covered by this Annual Report.

PART I.

Item 1.	Business	5
Item 1A.	Risk Factors	25
Item 1B.	Unresolved SEC Comments	49
Item 2.	Properties	49
Item 3.	Legal Proceedings	50
Item 4.	Mine Safety Disclosures	50

PART II.

Item 5.	Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	50
Item 6.	Selected Financial Data	51
Item 7.	Management's Discussion and Analysis of Financial Condition and Results of Operations	51
Item 7A.	Quantitative and Qualitative Disclosures about Market Risk	61
Item 8.	Financial Statements and Supplementary Data	62
Item 9.	Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	62
Item 9A.	Controls and Procedures	62
Item 9B.	Other Information	63

PART III

Item 10.	Directors, Executive Officers and Corporate Governance	64
Item 11.	Executive Compensation	64
Item 12.	Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	64
Item 13.	Certain Relationships and Related Transactions, and Director Independence	64
Item 14.	Principal Accountant Fees and Services	64

PART IV

Item 15.	Exhibits, Financial Statements and Schedules	65
Item 16.	Form 10-K Summary	68

Signatures	69
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PRESENTATION NOTE:

As previously disclosed, on January 4, 2018, Rocket Pharmaceuticals, Inc. (f/k/a Inotek Pharmaceuticals Corporation), a Delaware corporation (the "Company"), completed the reverse merger (the "Reverse Merger") of its wholly owned subsidiary, Rome Merger Sub ("Merger Sub"), with and into Rocket Pharmaceuticals, Ltd., a Cayman Islands exempted company ("Rocket Ltd"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among the Company, Merger Sub and Rocket Ltd (the "Merger Agreement"). As a result of the Reverse Merger, Rocket Ltd, the surviving company in the Reverse Merger, became a wholly owned subsidiary of the Company and, following the Reverse Merger, the Company changed its corporate name from Inotek Pharmaceuticals Corporation to Rocket Pharmaceuticals, Inc.

For accounting purposes, the Reverse Merger is treated as a "reverse acquisition" under generally acceptable accounting principles in the United States ("U.S. GAAP") and Rocket Ltd is considered the accounting acquirer. Accordingly, Rocket's Ltd's historical results of operations have replaced the Company's historical results of operations for all periods prior to the Reverse Merger.

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K 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 on Form 10-K 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, 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 successfully develop and commercialize any technology that we may in-license or products we may acquire;
- unanticipated delays due to manufacturing difficulties, 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 Form 10-K.

Any forward-looking statements in this Annual Report on Form 10-K 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 on Form 10-K. 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 on Form 10-K 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****Merger of Inotek Pharmaceuticals Corporation and Rocket Pharmaceuticals, Ltd.**

On January 4, 2018, Inotek Pharmaceuticals Corporation (“Inotek”) and privately held Rocket Pharmaceuticals, Ltd. (“Rocket Ltd”) completed a business combination in accordance with the terms of the Agreement and Plan of Merger and Reorganization (the “Merger Agreement”), dated as of September 12, 2017, by and among Inotek, Rome Merger Sub, a wholly owned subsidiary of Inotek (“Merger Sub”), and Rocket Ltd, pursuant to which Merger Sub merged with and into Rocket Ltd, with Rocket Ltd surviving as a wholly owned subsidiary of Inotek. This transaction is referred to as the “Reverse Merger.” Immediately following the Reverse Merger, Inotek changed its name to “Rocket Pharmaceuticals, Inc.” In connection with the closing of the Reverse Merger, our common stock began trading on The Nasdaq Global Market under the ticker symbol “RCKT” on January 5, 2018.

The Reverse Merger has been accounted for as a reverse merger under the acquisition method of accounting. After reviewing the relative voting rights, the composition of the board of directors and the composition of senior management of the combined company after the Reverse Merger, it was determined that Rocket Ltd will be treated as the accounting acquirer and Inotek will be treated as the “acquired” company for financial reporting purposes under the acquisition method of accounting.

Overview

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. We have 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 and Leukocyte Adhesion Deficiency-I (“LAD-I”), a genetic disorder that causes the immune system to malfunction. FA has been in clinical stage testing in the EU since 2016, and in the United States (“U.S.”), Rocket received investigational new drug (“IND”) clearance for both FA and LAD-I in late 2018. Two additional pre-clinical stage LVV programs include Pyruvate Kinase Deficiency (“PKD”), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia; and Infantile Malignant Osteopetrosis (“IMO”), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. In addition, we have an adeno-associated virus (“AAV”) program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. An IND filing was cleared in Danon disease in early 2019, which will permit the commencement of human clinical studies following approval. We have global commercialization and development rights to all of our product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program (described below) for which we currently only have development rights.

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 Novartis's treatment for pediatric acute lymphoblastic leukemia indicates that there is a regulatory pathway forward for gene therapy products.

Essential Terminology.

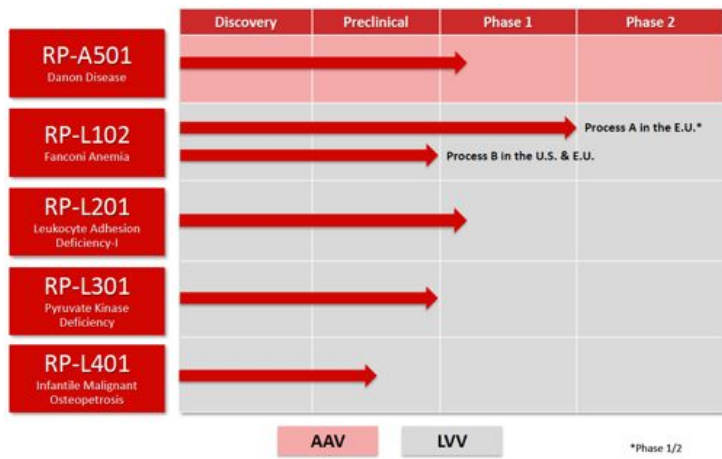
Set forth below is an abbreviated index of certain key terms and optimal ranges of values used in the discussion of LVV and AAV gene therapies.

Term	Definition	Optimal Ranges
<i>LVV Therapy (hematopoietic disorders)</i>		
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) [<i>in vivo</i> , 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 <i>in vivo</i> VCNs << 1.0
<i>AAV Therapy</i>		
Vector copy number (VCN) [<i>in vivo</i> , 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 <i>in vivo</i> VCNs << 1.0

Pipeline Overview

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.

The chart below shows the current phases of development of Rocket's programs and product candidates:



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

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 the current Rocket 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. Rocket’s 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. Rocket believes 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.

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, Rocket believes there are several critical factors that will lead Rocket's 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. Rocket believes 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. 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 Rocket's programs.* As a result, Rocket believes 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 Rocket's 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 Rocket's 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 optimization of future gene therapy clinical study design, with programs designed to overcome FA-specific gene therapy challenges. Rocket 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 Rocket's 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, Rocket's 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 of bone marrow failure or leukemia.

Rocket's 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 European Union ("EU") is estimated to be about 2,000, and given the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now thought to be in the 400-500 range, or at least double previous estimates.

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 a Rocket-sponsored Phase 1 clinical trial treating FA patients initially at the Center for Definitive and Curative Medicine at Stanford University School of Medicine (“Stanford”). A modified process under an Investigational Medicinal Product Dossier (“IMPD”), is being studied in a trial sponsored by CIEMAT. We are entitled to the data from this clinical study and have the commercial rights to the drug being studied under this IMPD.

At the American Society of Cell and Gene Therapy (“ASGCT”) Annual Meeting in May 2018, updated data from the ongoing Phase 1/2 clinical trial of RP-L102 sponsored by CIEMAT was presented, which included data from four patients that had been followed for 12 to 24 months and a fifth patient, treated with transduction-enhanced RP-L102, that was followed for two months. All patients demonstrated continued improvement in engraftment following administration of RP-L102 with sustained phenotypic reversals and earlier evidence of gene correction seen in higher-dosed patients. The progressive increases of corrected versus non-corrected peripheral blood leukocytes indicate the potential of RP-L102 to restore the functionality of bone marrow hematopoietic stem cells. The first patient that received transduction enhanced RP-L102 showed the highest transduction efficiency seen to date in all five patients treated, with a preliminary drug product vector copy number (“VCN”) of ~2.5 to 3, and a cell dose considered below the ideal target level of at least 500,000K CD34+/kg.

In June 2018, we were notified that the European Medicines Agency (“EMA”) classified RP-L102 as an Advanced Therapy Medicinal Product (“ATMP”). The ATMP classification recognizes and defines medicines for human use that are considered gene-, tissue-or cell-based therapies. The key benefit of ATMP classification is the early involvement and guidance from the EMA’s Committee of Advanced Therapies, which is the regulatory reviewing body for gene therapies in the EU.

In July 2018, we were notified that we received Rare Pediatric Disease designation from the FDA for RP-L102 for the treatment of FANCA. 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.

In August 2018, at our request, the Fred Hutchinson Cancer Center (“Hutch”), withdrew the IND for RP-L101, a program that we in-licensed from Hutch.

Additional FA data was presented at the October 2018 Annual Congress of European Society of Gene and Cell Therapy (“ESGCT”). At ESGCT, we presented clinical data from patients that had been followed for more than 12 months from the ongoing Phase 1/2 clinical trial of RP-L102 for FA under the first generation un-optimized process. The results continue to demonstrate improvement in engraftment with increased resistance to mitomycin-C and chromosomal stability of T-lymphocytes in the presence of diepoxybutane, two key diagnostic measures of functional and phenotypic correction of bone marrow cells. Data also showed evidence of stabilization of bone marrow failure in all four patients and progressive increases of corrected versus non-corrected peripheral blood leukocytes.

We submitted an IND to initiate clinical studies of RP-L102 in the U.S. in September 2018 and were notified by the FDA of its clearance in November 2018. In the second quarter of 2019, Rocket plans to treat patients in the U.S. under a Rocket-sponsored IND and in the EU with “Process B” RP-L-102. The planned clinical trial of RP-L102 is expected to enroll patients at Stanford beginning in March 2019. This new optimized process incorporates higher cell doses, transduction enhancers, and commercial-grade vector.

In November 2018, we were notified that the FDA granted Regenerative Medicine Advanced Therapy (“RMAT”), and Fast Track designations for RP-L102. RMAT designation was granted based on the positive efficacy and safety results from the ongoing Phase 1/2 clinical trial of RP-L102 being conducted in Europe.

Regulatory Status

The FA program in the EU is in the clinical-stage with an IMPD in place with the Spanish Agency for Medicines and Health Products. The program is also in the clinical stage in the U.S., with patients being enrolled in the first half of 2019. Both the FDA and the EMA have granted orphan drug designation for the “Lentiviral vector carrying the Fanconi anemia-A (FANCA) gene for the treatment of Fanconi anemia type A.”

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 (both living and deceased) 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 HSCT.

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), Rocket is 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.

Preclinical Proof of Concept

Preclinical results have indicated correction of LAD-I in mouse models, including restoration of neutrophils’ ability to adhere to endothelial surfaces and migrate from blood vessels towards inflammatory sources. Specifically, gene correction has been shown to restore functional CD18 expression in a CD18 hypomorphic mouse (CD18^{HYP}) model, in which a mouse is conferred a CD18 gene mutation resulting in impaired inflammatory responses, leukocytosis (high white blood cell count) and hepatosplenomegaly (swelling of the liver and spleen).

Rocket currently has one program targeting LAD-I, RP-L201. RP-L201 is a clinical program that Rocket in-licensed from CIEMAT. The planned open-label, single-arm, Phase 1 portion of the clinical trial of RP-L201 is expected to enroll two severe LAD-I patients in the U.S or E.U. The Phase 2 portion of the clinical trial of RP-L201 is expected to be a registration-enabling global study with clinical sites in the U.S., U.K. and Spain.

Regulatory Status

We submitted an IND to initiate clinical studies of RP-L201 in the U.S. in October 2018 and were notified by the FDA of its clearance in November 2018. In November 2018, we were notified that we received Rare Pediatric Disease designation and in December 2018 we received Fast Track designation from the FDA for RP-L201.

In January 2019, we were notified that the EMA, classified RP-L201 as an ATMP. We plan to initiate Rocket sponsored clinical studies of RP-L201 in 2019 in the U.S. and Spain.

In the EU, the IMPD was approved by Spanish Agency for Medicines and Health in November 2018.

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.

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.

We currently have one LVV-based program targeting PKD, RP-L301. RP-L301 is a preclinical program that we in-licensed from CIEMAT. This program is currently being developed through an ongoing collaboration with CIEMAT. New market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 per year.

Regulatory Status

In the EU, the PKD program has been discussed with the EMA via a Scientific Advisory meeting in 2016. This program has been granted EMA orphan drug disease designation and FDA orphan drug disease designation. A rolling IMPD is expected to be filed in the next few months. We expect to initiate a Phase 1 clinical trial of RP-L301 in the second half of 2019.

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

Rocket currently has one LVV-based program targeting IMO, RP-L401. RP-L401 is a preclinical program that Rocket in-licensed from Lund University, Sweden. This program has been granted ODD from the FDA.

AAV Program.

Overview of Danon Disease

Danon disease is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. RP-A501 is in preclinical development 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. 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.

Preliminary preclinical studies have indicated that clinically feasible AAV doses can restore functional levels of protein in knockout mouse models, and that gene/protein restoration is associated with marked histologic and hemodynamic improvement in the organs in which the disorder causes extensive morbidity and mortality. Rocket's AAV program is designed to enable a single-injection definitive therapy for this devastating disease, in which there exists no reliably curative treatment option.

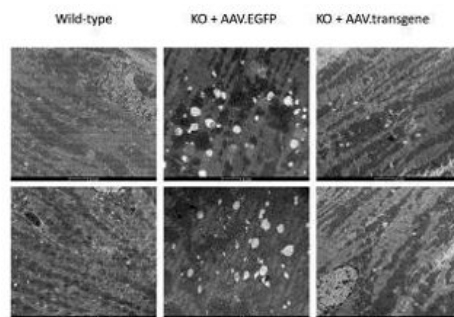


Figure 1

FIGURE 1: Representative electron microscopy tissue images from animal model of undisclosed AAV program. Left panels indicate wild-type (normal) animals; middle panels indicate diseased animals treated with control (“EGFP”) vector; and right panels indicate RP-A501 mediated restoration of architecture and resolution of additional abnormalities.

In November 2018, we announced our AAV-based RP-A501 program for the treatment of Danon disease, along with animal study data providing preclinical proof-of-concept for the RP-A501 program. Preclinical efficacy studies were performed in LAMP-2 knockout mice. Four doses of vector were tested for optimal transduction of the heart, skeletal muscle, and liver. Toxicology studies were conducted in wild-type mice and non-human primates. The results from these studies are summarized as follows:

- Increased survival rates were observed at higher doses of RP-A501 along with dose-dependent improvements and restoration of cardiac function.
- RP-A501 elicited phenotypic reversals at a structural and molecular level in cardiac, liver, and skeletal muscle tissue.
- There were no treatment-related adverse events or deaths associated with RP-A501. All doses were observed to be well-tolerated in Good Laboratory Practice biodistribution and toxicology studies in both wildtype mice and additional studies in non-human primates.

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

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. In the medium and long-term, we expect to develop proprietary in-house analytics and manufacturing capabilities, commence registration trials for our currently planned programs and 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

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

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

Rocket has in-licensed numerous patent applications and possesses substantial know-how and trade secrets relating to the development and commercialization of gene therapy products. Rocket's 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 1, 2019, Rocket's patent portfolio includes five in-licensed patent families relating to its product candidates and related technologies, discussed more fully below.

Fanconi Anemia

Rocket's Fanconi Anemia program includes two in-licensed patent families. The first family 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 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. Rocket expects 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)

Rocket's 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. 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 2037, absent any patent term adjustments or extensions.

Rocket's objective is to continue to expand its portfolio of patents and patent applications in order to protect Rocket's gene therapy product candidates and manufacturing processes. From time to time, Rocket may also evaluate opportunities to sublicense its portfolio of patents and patent applications that it owns or exclusively licenses, and Rocket 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 Rocket 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, Rocket expects to apply for patent term extensions for patents covering Rocket's product candidates and their methods of use.

Rocket may rely, in some circumstances, on trade secrets to protect its technology. However, trade secrets can be difficult to protect. Rocket seeks to protect its proprietary technology and processes, in part, by entering into confidentiality agreements with its employees, consultants, scientific advisors and third parties. Rocket also seeks to preserve the integrity and confidentiality of its data and trade secrets by maintaining physical security of its premises and physical and electronic security of its information technology systems. While Rocket has confidence in these individuals, organizations and systems, agreements or security measures may be breached, and Rocket may not have adequate remedies for any breach. In addition, Rocket's trade secrets may otherwise become known or be independently discovered by competitors. To the extent that Rocket's consultants or collaborators use intellectual property owned by others in their work for Rocket, 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, Rocket entered into an exclusive license agreement with Hutch granting Rocket 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.

As consideration for the licensed rights, in November 2015, Rocket issued to Hutch ordinary shares valued at \$0.3 million as an upfront license fee. Rocket is 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, Rocket is obligated to pay a low to mid-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 sublicenses in specified circumstances.

In December 2015, Rocket entered into an exclusive license agreement with Hutch granting Rocket 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 Rocket issued to Hutch ordinary shares valued at \$0.1 million as an upfront license that was expensed as research and development costs. Rocket is 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 Rocket enters into a sublicense agreement with a sublicensee, Rocket will be obligated to pay a portion of any consideration received from such sublicenses in specified circumstances.

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

As consideration for the licensed rights, Rocket paid CIEMAT an initial upfront license fee of €0.03 million (approximately \$0.03 million) which was expensed as research and development costs. Rocket is obligated to make aggregate milestone payments of up to €1.4 million (approximately \$1.5 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the PKD license, Rocket is obligated to pay a low to mid-single digit percentage royalty on net sales, subject to specified adjustments, by 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.

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

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

As consideration for the licensed rights, Rocket paid CIEMAT an initial upfront license fee of €0.1 million (approximately \$0.1 million), which was expensed as research and development costs. Rocket is obligated to make aggregate milestone payments of up to €5.0 million (approximately \$6.0 million) to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the license, Rocket is obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by Rocket or its sublicensees or affiliates. In the event that 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.

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

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 granted 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 renewed for an additional year expiring August 2019. Rocket may terminate this agreement at any time by providing Lund University and Dr. Richter with 90 days' advance notice.

License Agreement for LAD-I with CIEMAT and UCLB

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

As consideration for the licensed rights, Rocket paid Licensors an initial upfront license fee of €0.03 million (approximately \$0.04 million), which was expensed as research and development costs. Rocket is obligated to make aggregate payments of up to €1.4 million (approximately \$1.5 million) to Licensors upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the LAD-I license, Rocket is obligated to pay a mid-single digit percentage royalty on net sales, subject to specified adjustments, by 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.

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

License Agreement for Danon Disease

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 research and development 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, the Company through its wholly-owned subsidiary Rocket Pharmaceuticals, Ltd., entered into a License Agreement (the “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”).

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 which was expensed to R&D costs in the consolidated financial statement. 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 products incorporating the Licensed Patents (the “Licensed Products”) during the royalty term. If successful, the Company will be required to make milestone payments to RGNX of up to \$13.0 million for each Licensed Product upon the achievement of specified clinical development and regulatory milestones in the U.S. and EU. In addition, the Company shall pay RGNX 20% of the payment fees received from a priority review voucher issued in connection with or otherwise related to a Licensed Product. These royalty obligations are subject to specified reductions if additional licenses from third parties are required. The Company must also pay RGNX a portion of all non-royalty sublicense income (if any) received from sublicensees.

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 Rocket believes that its experience and scientific knowledge provides it with competitive advantages, Rocket faces 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 Rocket’s pipeline that have not yet been conceived. Any product candidates that Rocket successfully develops and commercializes will compete with existing therapies such as bone marrow transplantation and new therapies that may become available in the future. Rocket believes that the key competitive factors affecting the success of Rocket’s 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, Rocket intends to develop single treatment curative therapies for clinical indications that address mortality or high morbidity, which could differentiate Rocket 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 Rocket 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, Rocket’s programs.

Rocket anticipates that it will face intense and increasing competition as new drugs and therapeutic modalities enter the market and advanced technologies become available. Rocket’s commercial opportunity could be reduced or eliminated if Rocket’s 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 Rocket may develop. Rocket’s potential competitors also may obtain FDA or other regulatory approval for their products more rapidly than Rocket may obtain approval for its products.

Manufacturing

Rocket’s 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. Rocket does not currently operate manufacturing facilities for clinical or commercial production of its product candidates. In the future, Rocket may supplement or replace current supply arrangements with its own direct manufacturing capabilities. Rocket currently relies on third-party manufacturers to produce the plasmids, vectors, cell banks and final drug product for its clinical trials. Rocket manages such production with its vendors on a purchase order basis in accordance with applicable master service and supply agreements. Rocket has not yet entered into long-term agreements with these manufacturers or any other third-party suppliers, as it is customary in the industry to enter into commercial supply agreements upon either achievement of proof-of-concept or as a company approaches registration trials. Rocket, however, intends to procure quantities on a purchase order basis from redundant and multiple sources for Rocket’s clinical and commercial production to mitigate risk. If any of Rocket’s existing third-party suppliers should become unavailable to Rocket for any reason, Rocket believes that there are a number of potential replacements, although Rocket might experience a delay in its ability to obtain alternative suppliers. Rocket also does not have any current contractual relationships for the manufacture of commercial supplies of its product candidates if they become registered. With respect to commercial production of Rocket’s product candidates in the future, Rocket plans to pursue multiple options including outsourcing production of the active pharmaceutical (drug substance) ingredients as well as 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.

Rocket expects to continue to develop drug candidates that can be produced in a cost effective manner at contract manufacturing facilities. However, should a supplier or manufacturer on which Rocket has relied to produce a product candidate provide Rocket with a faulty product or such product is later recalled, Rocket 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 research and development 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 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 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 current Good Manufacturing Practice (“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 an NDA 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. Sponsors typically use the End-of-Phase 2 meetings to discuss their Phase 2 clinical results and present their plans for the pivotal Phase 3 trials that they believe will support approval of the new drug.

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 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.

- **Breakthrough therapy designation.** To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives: intensive guidance on an efficient drug development program; intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review; and rolling review.
- **Priority review.** A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious condition compared to marketed products. 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 products in accordance with cGMP regulations. 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 or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. Therefore, our products, once approved, may not obtain market acceptance unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products.

The process for determining whether a third-party payor will provide coverage for a drug product typically is separate from the process for setting the price of a drug product or for establishing the reimbursement rate that the payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, also known as a formulary, 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 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. A plan for the research will be developed by the U.S. 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.

Research and Development (“R&D”)

For the years ended December 31, 2018, 2017 and 2016, Rocket’s research and development expenses were \$53.3 million, \$14.9 million and \$6.0 million, respectively.

Employees

We had 33 full-time employees as of March 1, 2019, and an additional 6 consultants responsible for critical functions. 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 our 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 on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after we electronically file such material with, or furnish it to, the Securities and Exchange Commission (“SEC”). Our SEC reports can be accessed through the Investors section of our website. The SEC maintains a website that contains reports, proxy and information statements and other information regarding our filings at www.sec.gov. The information found on our website is not incorporated by reference into this report or any other report we file with or furnish to the SEC. Our common stock is listed on the Nasdaq Global Market under the symbol “RCKT.”

Item 1A. Risk Factors

We operate in an industry that involves numerous risks and uncertainties. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Annual Report on Form 10-K, 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 and conducting preclinical research and development 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 \$74.5 million, \$19.6 million and \$7.6 million for the years ended December 31, 2018, 2017 and 2016, respectively. As of December 31, 2018, we had an accumulated deficit of \$105.9 million. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development 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 research and development, clinical testing, regulatory compliance activities, 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. In addition, 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. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2018, our cash, cash equivalents and investments was \$213.1 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; and
- 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 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.

In addition, as a relatively new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We are still in the early stages of transitioning from a drug-discovery company with a licensing and research focus to a clinical-stage company that is supporting clinical development activities, and we may need to transition to supporting commercial activities in the future. We cannot guarantee that we will be successful in these transitions.

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 are able to 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 therapy products have been approved in the United States and the European Union.

We have concentrated our research and development 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 therapy products have received marketing authorization in the U.S. or the EU, including Novartis Pharmaceuticals' Kymriah, 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. Our clinical programs to date have been performed under an IMPD, in Spain sponsored by CIEMAT, and in the U.S. sponsored by Hutch. The clinical trials performed by these sponsors were for a lentiviral treatment for FA, a rare mutation of the FANC-A gene. We have now initiated Rocket-sponsored clinical trials for FA and LAD-I, 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; 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.

Moreover, we intend to rely on the nonclinical studies and clinical trials performed by CIEMAT, and the FDA or the regulatory authority in any other country in which we decide to perform clinical trials or seek approval may not accept that results of the CIEMAT studies and trials. Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties.

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 a sufficient number of 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 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.

Our FA gene therapy treatment was studied in clinical trials conducted by our partners. We have now initiated Rocket sponsored clinical trials for FA and LAD-I, 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 programs are in the preclinical stages. 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 more narrow 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 relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, 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, 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 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.

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 March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA, was signed into law, and 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. States 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 research and development 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 research and development 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 research and development 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. Research and development was our most significant operating expense for the year ended December 31, 2018. Research and development 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 research and development 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 research and development 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 all of 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 or not 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 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 research and development 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 solely on third-party manufacturers to manufacture supplies of our product candidates. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured 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 all 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 research and development 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 does 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 1, 2019, we had 33 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 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.

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, 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.

Affordable Care Act and Other Reform Initiatives

In the United States and some foreign jurisdictions, there have been, and likely will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system directed at broadening the availability of healthcare and containing or lowering the cost of healthcare.

By way of example, in March 2010, the ACA was enacted. The ACA includes measures that have or will significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA of greatest importance to the pharmaceutical industry are the following:

- The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the U.S. Department of Health and Human Services in exchange for state Medicaid coverage of most of the manufacturer’s drugs. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers’ rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs and biologic agents to 23.1% of average manufacturer price (“AMP”) and adding a new rebate calculation for “line extensions” (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP.
- The ACA expanded the types of entities eligible to receive discounted 340B pricing, although, with the exception of children’s hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs used in orphan indications. In addition, because 340B pricing is determined based on AMP and Medicaid drug rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discounts to increase. The ACA imposed a requirement on manufacturers of branded drugs and biologic agents to provide a 50% discount off the negotiated price of branded drugs dispensed to Medicare Part D beneficiaries in the coverage gap (i.e., “donut hole”).
- The ACA imposed an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications.
- The ACA included the Federal Physician Payments Sunshine Act, which requires certain pharmaceutical manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children’s Health Insurance Program, with specific exception, to track certain financial arrangements with physicians and teaching hospitals, including any “transfer of value” provided, as well as any ownership or investment interests held by physicians and their immediate family members. Covered manufacturers were required to begin collecting data on August 1, 2013 and submit reports on aggregate payment data to CMS for the first reporting period (August 1, 2013—December 31, 2013) by March 31, 2014, and were required to report detailed payment data for the first reporting period and submit legal attestation to the completeness and accuracy of such data by June 30, 2014. Thereafter, covered manufacturers must submit reports by the 90th day of each subsequent calendar year. The information reported was made publicly available on a searchable website in September 2014.
- The ACA established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products.
- The ACA created the Independent Payment Advisory Board which has the authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. Under certain circumstances, these recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings.
- The ACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to improve quality of care and lower program costs of Medicare, Medicaid and the Children’s Health Insurance Program, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation through 2019.

Many of the details regarding the implementation of the ACA are yet to be determined, and at this time, it remains unclear the full effect that the ACA will have on our business.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

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 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 trials legislation is currently undergoing a revision process mainly 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.

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

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system 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 a number of 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 Affordable Care Act, 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 Affordable Care Act or otherwise circumvent some of the requirements for health insurance mandated by the Affordable Care Act. One Executive Order directs federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act 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 Affordable Care Act. 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 Affordable Care Act risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the Affordable Care Act marketplace, providers, and potentially our business, are not yet known.

In July 2018, the CMS published a final rule permitting further collections and payments to and from certain Affordable Care Act-qualified health plans and health insurance issuers under the Affordable Care Act 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 has recently published a final rule that would give 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 Affordable Care Act for plans sold through such marketplaces. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseparable feature of the Affordable Care Act, and therefore, because it was repealed as part of the Tax Cuts and Jobs Act of 2017, the remaining provisions of the Affordable Care Act are invalid as well. While the Trump Administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business.

Since its enactment, some of the provisions of the Affordable Care Act 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 Affordable Care Act. 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 has been 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 Affordable Care Act-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, or the BBA, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.”

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act 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. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. 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 September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. 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.

The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize any products for which we obtain marketing approval.

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.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

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 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 have an effect on 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.

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 all of 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 research and development 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 consists primarily of 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 licensees 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 that we may not be able 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 expire from 2019 to 2023, if our products were to launch before these dates, 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 to 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 purchase our product candidates and 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. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

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.

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 experiences 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.

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

On September 16, 2011, the Leahy-Smith America Invents Act (the “Leahy-Smith Act”), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, were enacted March 16, 2013. It is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. 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 and financial condition.

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 U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, 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 it 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

A significant number of our total outstanding shares may be sold into the market in the near future, which 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.

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 principal 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 36.50% 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.

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 of our 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 on Form 10-K 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.

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 could be an emerging growth company until December 31, 2020, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds \$700 million as of any June 30 before that time, in which case we would no longer be an emerging growth company as of the following December 31. 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 May 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.

In August 2018, we entered into a lease for approximately 92,000 rentable square feet in Cranbury, New Jersey, for internal process development and research activities to support our pipeline, this lease 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

On January 6, 2017, a purported stockholder of Inotek filed a putative class action in the U.S. District Court for the District of Massachusetts, captioned *Whitehead v. Inotek Pharmaceuticals Corporation, et al.*, No. 1:17-cv-10025. An amended complaint was filed on July 10, 2017, and a second amended complaint was filed on September 5, 2017. The second amended complaint alleged violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and SEC Rule 10b-5 against Inotek, David Southwell, and Rudolf Baumgartner based on allegedly false and misleading statements and omissions regarding Inotek's phase 2 and phase 3 clinical trials of *trabodenson*. The lawsuit sought, among other things, unspecified compensatory damages for purchasers of Inotek's common stock between July 23, 2015 and July 10, 2017, as well as interest and attorneys' fees and costs. The second amended complaint was dismissed with prejudice on June 27, 2018. Plaintiffs filed a notice of appeal to the First Circuit Court of Appeals on July 27, 2018, voluntarily dismissed their appeal on August 31, 2018, and the First Circuit entered judgment dismissing the action on September 5, 2018.

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 1, 2019, there were 22 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

On December 28, 2018, we sold 100,000 shares of common stock to RTW Innovation Master Fund, Ltd. for an aggregate offering price of approximately \$1.4 million, including reimbursement for our expenses incurred in connection with the transaction. The offering and sale was completed as a private placement under Section 4(a)(2) of the Securities Act of 1933, as amended.

Issuer Purchases of Equity Securities

The following table provides information about purchases by the Company of its shares of common stock during the three-month period ended December 31, 2018. The purchases were not made under any publicly announced plans or programs.

	(a) Total Number of Shares Purchased ⁽¹⁾	(b) Average Price Paid per Share	(c) Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	(d) Maximum Number of Shares that May Yet be Purchased Under the Plans or Programs
October 1-31, 2018	—	—	—	—
November 1-30, 2018	—	—	—	—
December 1-31, 2018	100,000	\$ 13.93	—	—

(1) Our Board of Directors approved the repurchase of 100,000 shares of our common stock, par value \$0.01 per share, which was outside of a publicly-announced repurchase plan. 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 by the Company settled on December 31, 2018 and the remaining 50,000 shares repurchased settled on January 2, 2019

Item 6. Selected Financial Data

The following selected consolidated financial data should be read in conjunction with Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and the consolidated financial statements and the notes thereto included elsewhere in this report. The selected consolidated financial data in this section are not intended to replace our consolidated financial statements and the related notes. Our historical results are not necessarily indicative of our future results.

	For the Years Ended December 31,			Period from
	2018	2017	2016	July 14 to December 31, 2015
Consolidated Statements of Operations Data:				
Operating expenses:				
Research and development	\$ 53,270	\$ 14,917	\$ 5,994	\$ 3,236
General and administrative	17,886	4,855	1,580	184
Loss from operations	(71,156)	(19,772)	(7,574)	(3,420)
Research and development incentives	186	192	-	-
Interest expense	(6,039)	-	-	(7)
Interest and other income net	1,690	2	1	1
Accretion of discount on investments	801	-	-	-
Loss on debt conversion	-	-	-	(778)
Net loss	\$ (74,518)	\$ (19,578)	\$ (7,573)	\$ (4,204)
Net loss per common share—basic and diluted	\$ (1.89)	\$ (2.88)	\$ (1.11)	\$ (0.62)
Weighted-average common shares outstanding—basic and diluted	39,377,666	6,795,627	6,833,718	6,833,718

	As of December 31,	
	2018	2017
Consolidated Balance Sheet Data:		
Cash, cash equivalents and investments	\$ 213,132	\$ 18,142
Total assets	251,313	20,147
Convertible notes	41,447	-
Total liabilities	57,276	4,628
Accumulated deficit	(105,873)	(31,355)
Total shareholders’ equity	194,037	15,519

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 “Selected Financial Data” and our consolidated financial statements, related notes and other financial information included elsewhere in this Annual Report on Form 10-K. 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 on Form 10-K.

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 after the reverse merger described herein. 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 on Form 10-K, 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. We have clinical-stage 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 and Leukocyte Adhesion Deficiency-I (“LAD-I”), a genetic disorder that causes the immune system to malfunction. FA has been in clinical stage testing in the EU since 2016, and in the U.S., Rocket received investigational new drug (“IND”) clearance for both FA and LAD-I in late 2018. Two additional pre-clinical stage LVV programs include Pyruvate Kinase Deficiency (“PKD”), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia; and Infantile Malignant Osteopetrosis (“IMO”), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. In addition, we have an AAV program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. An IND filing was cleared in Danon disease in early 2019, which will permit the commencement of human clinical studies following approval. We have global commercialization and development rights to all of our product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program for which we currently only have development rights.

Recent Developments

Reverse Merger and Exchange Ratio

On January 4, 2018, Rome Merger Sub (“Merger Sub”), a wholly owned subsidiary of Inotek, completed its merger with and into Rocket Ltd, with Rocket Ltd surviving as a wholly owned subsidiary of Inotek. This transaction is referred to as the “Reverse Merger.” As a result of the Reverse Merger, each outstanding share of Rocket Ltd share capital (including shares of Rocket Ltd share capital to be issued upon exercise of outstanding share options) automatically converted into the right to receive approximately 76.185 shares of Inotek’s common stock, par value \$0.01 per share (the “Exchange Ratio”). Following the closing of the Reverse Merger, holders of Inotek’s common stock immediately prior to the Reverse Merger owned approximately 18.643% on a fully diluted basis, and holders of Rocket Ltd common stock immediately prior to the Reverse Merger owned approximately 81.357% on a fully diluted basis, of Rocket Ltd’s common stock.

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 Company’s common stock remained listed on the NASDAQ Stock Market, with trading having commenced on a post-split basis and under the new name as of January 5, 2018. The trading symbol also changed on that date from “ITEK” to “RCKT.”

On January 24, 2018, the Company entered into an underwriting agreement with Cowen and Company, LLC and Evercore Group L.L.C., as representatives (the “Representatives”) of the several underwriters (collectively with the Representatives, the “Underwriters”), pursuant to which the Company sold 6,325,000 shares of common stock (the “Shares”), which includes 825,000 shares that were sold pursuant to an option granted to the Underwriters (the “Offering”). The Shares were sold in the Offering at a public offering price of \$13.25 per share in which the Company received gross proceeds of \$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, after deducting the underwriting discounts and commissions and legal and accounting costs.

On May 16, 2018, Rocket and Stanford 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 were paid during the year ended December 31, 2018. As of December 31, 2018, none of the remaining milestones were met with regard to the LCGM. In January 2019, the Company and Stanford signed a Clinical Trial Agreement for the treatment of FA. Upon the signing of the Stanford Clinical Trial Agreement in January 2019, the second milestone of \$1.4 million for the LCGM became due.

On August 14, 2018, Rocket entered into a lease for approximately 92,000 rentable square feet in Cranbury, New Jersey, for office space, process development, research activities and potentially manufacturing to support the Company's pipeline (the "NJ Lease Agreement"). The term of the NJ Lease Agreement will commence for 72,000 rentable square feet upon substantial completion of leasehold improvements (the "Commencement Date"), and the remaining 20,000 square feet will commence upon the earlier of the Company's election to commence the lease of such additional space or thirty months from the Commencement Date. The NJ Lease Agreement has a term of fifteen years from the Commencement Date, with an option to renew for two consecutive five-year renewal terms. Estimated rent payments are \$1.2 million per annum, payable in monthly installments, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately \$26.5 million over the 15 year term of the lease. The Company delivered 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.

On November 19, 2018, the Company through our wholly-owned subsidiary Rocket Pharmaceuticals, Ltd., entered into a License Agreement (the "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"). 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").

In consideration for the rights granted to the Company under the License Agreement, we made an upfront payment to RGNX of \$7.0 million. 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, 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.

On November 30, 2018, we announced the closing of 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 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. BofA Merrill Lynch, Cowen and Company, LLC and Evercore Group, LLC acted as joint book-running managers for the offering. Oppenheimer & Co. acted as lead manager and Ladenburg Thalmann acted as co-manager.

In addition to the shares sold in the public offering, on November 30, 2018, Rocket closed 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, 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 December 27 and 28, 2018, we repurchased 100,000 shares of its common stock, par value \$0.01 per share, 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. Immediately following the foregoing stock repurchases (the "Repurchases"), we sold 100,000 shares of common stock (the "Shares") to RTW Innovation Master Fund, Ltd. ("RTW Innovation"), which is an affiliate of Rocket 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.

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, research and development activities for the programs 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, 2018, Rocket raised net cash proceeds of approximately \$195.2 million from investors through both equity and convertible debt financing to fund operating activities, including proceeds from the January 2018 and November 2018 public offerings.

Research and Development Expenses

Our research and development 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 research and development activities including, process development, preclinical, and clinical activities on Rocket's 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; 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 research and development activities are recorded as prepaid expenses.

Our direct research and development 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 research and development 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 research and development 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 share-based compensation, for our scientific personnel performing research and development 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 research and development activities.

Our research and development 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 research and development expenses will increase substantially over the next several years as we increase personnel costs, including share-based compensation, supports ongoing clinical studies, seeks to achieve proof-of-concept in one or more product candidates, advances preclinical programs to clinical programs, and prepares 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 research and development 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 research and development expenses to increase for the foreseeable future as we continue to invest in research and development 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 research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Our future research and development 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 research and development 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 research and development 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.

Rocket General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefit costs for personnel, including share-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, patents, 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 we will 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 are due in August 2021.

Interest Income

Interest income is related to interest earned from investments.

Results of Operations

Comparison of the Years Ended December 31, 2018 and 2017

The following table summarizes our results of operations for the years ended December 31, 2018 and 2017:

	For the Years Ended December 31,		
	2018	2017	Change
Operating expenses:			
Research and development	\$ 53,270	\$ 14,917	\$ 38,353
General and administrative	17,886	4,855	13,031
Total operating expenses	<u>71,156</u>	<u>19,772</u>	<u>51,384</u>
Loss from operations	(71,156)	(19,772)	(51,384)
Research and development incentives	186	192	(6)
Interest expense	(6,039)	-	(6,039)
Interest and other income net	1,690	2	1,688
Accretion of discount on investments	801	-	801
Total other income (expense) net	<u>(3,362)</u>	<u>194</u>	<u>(3,556)</u>
Net loss	<u>\$ (74,518)</u>	<u>\$ (19,578)</u>	<u>\$ (54,940)</u>

Research and Development Expenses

R&D expenses increased \$38.4 million to \$53.3 million for the year ended December 31, 2018 compared to the year ended December 31, 2017. The increases in R&D expenses were primarily a result of increases in manufacturing and process development expenses of \$22.3 million, an increase in license fees of \$6.9 million, an increase in R&D share-based compensation expense of \$6.9 million and an increase in compensation expense of \$2.4 million due to increased headcount.

General and Administrative Expenses

G&A expenses increased \$13.0 million to \$17.9 million for the year ended December 31, 2018 compared to the year ended December 31, 2017. The increase in G&A expenses was primarily due to merger-related expenses of \$5.3 million, including \$3.5 million of share-based compensation expenses and post-Reverse Merger transition expenses including payroll and severance payments for remaining Inotek employees retained for the post-Reverse Merger transition of \$1.9 million. The remaining increase of \$7.7 million is primarily due to increase in stock compensation expense of \$2.0 million, a \$3.7 million increase in personnel costs from headcount additions, and an increase in legal costs of \$0.7 million, insurance costs of \$0.7 million and filing fees of \$0.3 million in connection with supporting the growth in our business and becoming a public company in 2018. We expect an increase in general and administrative expenses in future periods, as we continue to operate as a public company.

Other Expense

Other expense was \$3.4 million for the year ended December 31, 2018 compared to other income of \$0.2 million for the year ended December 31, 2017. The movement was primarily due to an increase in interest expense of \$6.0 million, offset by an increase in interest income and accretion of discount on investments of \$2.5 million. The increase in interest expense is due to the assumption of the 2021 Convertible Notes in connection with the Reverse Merger. The increase in interest income is due to interest earned on the Company's investments.

Comparison of the Years Ended December 31, 2017 and 2016

The following table summarizes our results of operations for the years ended December 31, 2017 and 2016:

	For the Years Ended December 31,		
	2017	2016	Change
Operating expenses:			
Research and development	\$ 14,917	\$ 5,994	\$ 8,923
General and administrative	4,855	1,580	3,275
Total operating expenses	<u>19,772</u>	<u>7,574</u>	<u>12,198</u>
Loss from operations	(19,772)	(7,574)	(12,198)
Research and development incentives	192	-	192
Interest and other income net	2	1	1
Total other income net	<u>194</u>	<u>1</u>	<u>193</u>
Net loss	<u>\$ (19,578)</u>	<u>\$ (7,573)</u>	<u>\$ (12,005)</u>

Research and Development Expenses

R&D expenses increased \$8.9 million to \$14.9 million for the year ended December 31, 2017 compared to the year ended December 31, 2016. The increases were primarily a result of increases in manufacturing and process development expenses of \$4.7 million, increase in research expense of \$1.2 million and an increase in R&D share-based compensation expense of \$0.3 million and increase in compensation expense due to increased headcount of \$0.8 million for the year ended December 31, 2017 as compared to the year ended December 31, 2016.

General and Administrative Expenses

G&A expenses increased \$3.3 million to \$4.9 million for the year ended December 31, 2017 compared to the year ended December 31, 2016. The increase in G&A expenses was primarily due to an increase in G&A stock compensation expense of \$0.7 million, a \$1.1 million increase in personnel costs due to headcount additions, and an increase in merger related costs of \$1.0 million due to the Reverse Merger with Inotek.

Other Income

Other income was \$0.2 million for the year ended December 31, 2017 which consisted of the refund from the NYC Biotech tax credit.

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 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 drug 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. Rocket has incurred net losses and negative cash flows from its operations each year since inception. Rocket incurred net losses of \$74.5 million, \$19.6 million and \$7.6 million for the years ended December 31, 2018, 2017 and 2016, respectively. The Company has experienced negative cash flows from operations and has an accumulated deficit of \$105.9 million as of December 31, 2018. As of December 31, 2018, the Company has \$213.1 million of cash, cash equivalents and investments. Rocket expects such resources would be sufficient to fund its operating expenses and capital expenditure requirements into the second half of 2020. Rocket has funded its operations primarily through the sale of its equity and debt securities.

On January 26, 2018, Rocket closed a public offering of common stock of 6,325,000 shares of common stock and received net proceeds of approximately \$78.5 million. On November 30, 2018, the Company closed a public offering of 4,082,500 shares of its common stock and received gross proceeds of \$63.3 million. In addition to the shares sold in the public offering on November 30, 2018, Rocket closed a concurrent sale of 967,742 shares of common stock for gross proceeds of approximately \$15.0 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. 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.

Contractual Obligations

The following table summarizes our contractual obligations at December 31, 2018:

	<u>Total</u>	<u>Less than 1 year</u>	<u>1 to 3 years</u>	<u>3 to 5 years</u>	<u>More than 5 years</u>
Operating lease obligations	\$ 28,664	\$ 1,372	\$ 3,794	\$ 3,354	\$ 20,144
2021 Convertible Notes	52,000	-	52,000	-	-
Total	\$ 80,664	\$ 1,372	\$ 55,794	\$ 3,354	\$ 20,144

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 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.

Cash Flows

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

	For the Years Ended December 31,		
	2018	2017	2016
Cash used in operating activities	\$ (53,788)	\$ (15,962)	\$ (5,503)
Cash used in investing activities	(5,272)	(760)	(335)
Cash provided by financing activities	153,502	25,406	16
Net change in cash, cash equivalents and restricted cash	<u>\$ 94,442</u>	<u>\$ 8,684</u>	<u>\$ (5,822)</u>

Net Cash Used in Operating Activities

During the year ended December 31, 2018, operating activities used \$53.7 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 share-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.

During the year ended December 31, 2017, operating activities used \$16.0 million of cash, primarily resulting from Rocket's net loss of \$19.6 million and net changes in our operating assets and liabilities of \$2.1 million, partially offset by net non-cash charges of \$1.5 million, including share-based compensation expense of \$1.3 million. Changes in Rocket's operating assets and liabilities for the year ended December 31, 2017 consisted of a \$2.8 million increase in accounts payable and accrued expenses, offset by an increase in prepaid expenses and other assets of \$0.7 million.

During the year ended December 31, 2016, operating activities used \$5.5 million of cash, primarily resulting from Rocket's net loss of \$7.6 million and net changes in our operating assets and liabilities of \$1.5 million, partially offset by net non-cash charges of \$0.5 million, including share-based compensation expense of \$0.3 million. Changes in Rocket's operating assets and liabilities for the year ended December 31, 2016 consisted of a \$1.4 million increase in accounts payable and accrued expenses and a decrease in prepaid expenses and other current assets of \$0.1 million.

Net Cash Used in Investing Activities

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.

During the year ended December 31, 2017, Rocket used \$0.8 million of cash in investing activities, consisting primarily of purchases of property and equipment.

During the year ended December 31, 2016, Rocket used \$0.3 million of cash in investing activities, consisting of purchases of property and equipment.

Net Cash Provided by Financing Activities

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 closed a public offering of common stock and received net proceeds of approximately \$78.5 million. On November 30, 2018, we closed a public offering of 4,082,500 shares of its common stock. The gross proceeds to Rocket from the offering were approximately \$63.3 million. In addition to the shares sold in the public offering, Rocket closed a concurrent sale of 967,742 shares of common stock for gross proceeds of approximately \$15.0 million.

During the year ended December 31, 2017, net cash provided by financing activities was \$25.4 million, consisting of proceeds from Rocket's issuance of Series B convertible preferred shares. Net cash provided by financing activities was immaterial for the year ended December 31, 2016.

Operating Capital Requirements

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales, marketing and manufacturing. Accordingly, we anticipate that we will need substantial additional funding in connection with our continuing operations.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we are able to raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could cause potential dilution. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or research programs or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

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

Under Section 107(b) of the Jumpstart Our Business Startups Act of 2012 (the “JOBS Act”), an “emerging growth company” can delay the adoption of new or revised accounting standards until such time as those standards would apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, as a result, we will adopt new or revised accounting standards at the same time as other public companies that are not emerging growth companies. There are other exemptions and reduced reporting requirements provided by the JOBS Act that we are currently evaluating. For example, as an emerging growth company, we are exempt from Sections 14A(a) and (b) of the Exchange Act which would otherwise require us to (i) submit certain executive compensation matters to stockholder advisory votes, such as “say-on-pay,” “say-on-frequency” and “golden parachutes” and (ii) disclose certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of our Chief Executive Officer’s compensation to our median employee compensation. We also intend to rely on an exemption from the rule requiring us to provide an auditor’s attestation report on our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and the rule requiring us to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board (“PCAOB”) regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the consolidated financial statements as the auditor discussion and analysis. We will continue to remain an “emerging growth company” until the earliest of the following: December 31, 2020; the last day of the fiscal year in which our total annual gross revenue is equal to or more than \$1.07 billion; the date on which we have issued more than \$1 billion in nonconvertible debt during the previous three years; or the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Critical Accounting Policies and Estimates

Goodwill

Business combinations are accounted for under the acquisition method (see Note 4). 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. As amended, 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. Entities should apply for the amendment prospectively. We early adopted this guidance as of January 1, 2018. 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 year ended December 31, 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 conduct 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 research and development 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.

Rocket's material financial instruments at December 31, 2018 and 2017, consisted of cash and cash equivalents, investments, accounts payable and the 2021 Notes. The fair value of Rocket's cash and cash equivalents and accounts payable approximate their respective carrying values due to the short-term nature of these instruments and amounts. Rocket has determined that its money market mutual funds and U.S. Treasury securities are categorized as Level 1 assets under the fair value hierarchy, as these assets are valued using quoted market prices in active markets without any valuation adjustments.

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

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842), as amended, which supersedes the current leasing guidance and upon adoption, will require lessees to recognize right-of-use assets and lease liabilities on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. The new standard is effective for the Company for the annual period beginning after December 15, 2018. Upon the adoption of the guidance, operating leases are capitalized on the balance at the present value of lease payments. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 must be calculated using the applicable incremental borrowing rate at the date of adoption. See Note 13 for commitment and contingencies footnote for operating leases.

The FASB issued ASU 2018-11 in July 2018, which includes certain amendments to ASU 2016-02 intended to provide relief in implementing the new standard. Among these amendments is the option to not restate comparative periods presented in the financial statements. The Company intends to elect this new transition approach, using a cumulative-effect adjustment on the effective date of the standard, with comparative periods presented in accordance with the previous guidance in ASC 840. The Company currently expects to elect 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.

In preparation for adoption of the standard, we are in the process of implementing internal controls to enable the preparation of financial information including the assessment of the impact of the standard. The adoption of the new standard is expected to result in the recognition of additional lease liabilities ranging from \$3.0 million to \$3.7 million, and right-of-use assets ranging from \$2.6 million to \$3.2 million as of January 1, 2019 related to the Company's operating leases.

In January 2017, the FASB issued ASU 2017-01, Business Combinations (Topic 805): Clarifying the Definition of a Business ("ASU 2017-01"). ASU 2017-01 provides that when substantially all of the fair value of the assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the set is not a business. ASU 2017-01 is effective to annual period beginning after December 31, 2018 and interim period within annual periods beginning after December 31, 2019. The adoption of ASU 2017-09 may impact the Company's accounting for future acquisitions.

Item 7A. Quantitative and Qualitative Disclosures about Market Risks

As of December 31, 2018, Rocket had market risk exposure related to interest rate fluctuations. Rocket had cash, cash equivalents and investments of \$213.1 million at December 31, 2018, consisting primarily of funds in money market accounts, and U.S. Treasury securities. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the short-term nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows. Our 2021 Convertible Notes accrue interest at a fixed rate and therefore a change in interest rates would not impact the amount of interest we would have paid on this indebtedness.

Item 8. Financial Statements and Supplementary Data

The financial statements required by this Item 8 are included in Item 15 of this Annual Report on Form 10-K.

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Annual Report on Form 10-K, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of December 31, 2018, our principal executive officer and principal financial 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 officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Management’s Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over 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 assessed the effectiveness of our internal control over financial reporting as of December 31, 2018 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, 2018, our internal control over financial reporting is effective based on those criteria.

This annual report on Form 10-K 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 (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) 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

In October 2018, the Company entered into Severance and Change in Control Agreements (the “Severance Agreements”) with the following executives: Gaurav Shah, M.D., CEO and President, Kinnari Patel, Pharm D, MBA, EVP, COO and Head of Development, Jonathan Schwartz, M.D., CMO and Head of Clinical Development.

The Severance Agreements provide the executives with certain severance benefits. In connection with a termination of the executive’s employment by the Company for Cause or by the executive without Good Reason, the executive is entitled to receive (i) any accrued but unpaid base salary and accrued but unused vacation, (ii) any unpaid annual bonus with respect to any completed fiscal year immediately preceding the date of termination (provided, however, that if the executive is terminated for Cause, such bonus shall be forfeited), (iii) reimbursement for unreimbursed business expenses, and (iv) employee benefits, if any, to which the executive may be entitled as of the date of termination (i) through (iv) collectively, the “Accrued Amounts”).

In connection with a termination of the executive’s employment with the Company without Cause or by the executive for Good Reason, the executive is entitled to receive the Accrued Amounts and, upon execution of an irrevocable release of claims in favor of the Company, (i) a lump sum payment equal to 9 to 12 months of the executive’s annual base salary for the year in which termination occurs and (ii) if timely elected, 9 to 12 months of Company-paid COBRA benefits.

In connection with a termination of the executive’s employment with the Company without Cause or by the executive for Good Reason within 12 months following a Change in Control, the executive is entitled to receive, upon execution of an irrevocable release of claims in favor of the Company, (i) the Accrued Amounts, (ii) a lump sum payment equal to 12 to 18 months of the executive’s annual base salary for the year in which termination occurs, (iii) a lump sum amount equal to any annual bonus to which the executive would have been entitled for the year in which termination occurs, and (iv) if timely elected, 12 to 18 months of Company-paid COBRA benefits.

In connection with a termination of the executive’s employment upon death or disability of the executive, the executive (or the executive’s estate or beneficiaries) is entitled to receive the Accrued Amounts and a pro-rata portion of the annual bonus, if any, the executive would have earned for the year in which termination occurs.

For purposes of the Severance Agreements, “Cause” means (as determined by the Board in their discretion exercised in good faith):

- any material breach by the executive of any agreement between the executive and the Company;
- the conviction of, indictment for or plea of nolo contendere by the executive to a felony or a crime involving moral turpitude; or
- any material misconduct or willful and deliberate nonperformance (other than by reason of the executive’s disability) by the executive of the executive’s duties to the Company.

For purposes of the Severance Agreements, “Good Reason” means the occurrence of any of the following in each case during the term of employment without the executive’s written consent, which circumstances are not remedied by the Company within 30 days of its receipt of a written notice from the executive describing the applicable circumstances (which notice must be provided by the executive within 90 days of the executive’s knowledge of the applicable circumstances):

- a material, adverse change in the executive’s duties, responsibilities, authority, title or reporting structure;
- a material reduction in the executive’s base salary or bonus opportunity; or
- a geographical relocation of the executive’s principal office location by more than 50 miles.

For purposes of the Severance Agreements, a “Change in Control” shall be deemed to have occurred upon the occurrence of any one of the following events:

- the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity;
- a merger, reorganization or consolidation pursuant to which the holders of the Company’s outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or other equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction;
- the sale of all of the stock of the Company to an unrelated person, entity or group thereof acting in concert; or
- any other transaction in which the owners of the Company’s outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

The foregoing summary of Severance Agreements does not purport to be complete and is qualified in its entirety by reference to the complete Form of Change in Control and Severance Agreement, which is attached as Exhibit 10.19.

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 2018 Annual Meeting of Stockholders (“Proxy Statement”) or an amendment to this Annual Report on Form 10-K (“Form 10-K/A”) under the headings “Election of Directors,” “Executive Officers,” “Section 16(a) Beneficial Ownership Reporting Compliance” and “Corporate Governance” and is incorporated herein by reference. The Proxy Statement or Form 10-K/A will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

We have adopted a Code of Business Conduct and Ethics that applies to all of our directors, officers and employees, including our principal executive, principal financial and principal accounting officers or persons performing similar functions. Our Code of Business Conduct and Ethics is posted on our website located at www.rocketpharma.com. We intend to disclose any future amendments to certain provisions of the Code of Business Conduct and Ethics, and any waivers of the Code of Business Conduct and Ethics granted to executive officers and directors, on our website within four business days following the date of amendment or waiver.

Item 11. Executive Compensation

Information with respect to this item will be set forth in the Proxy Statement or Form 10-K/A under the headings “Executive Compensation” and “Director Compensation” and is incorporated herein by reference. The Proxy Statement or Form 10-K/A 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 or Form 10-K/A 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 or Form 10-K/A 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 or Form 10-K/A under the headings “Transactions with Related Persons” and “Information about the Board and Corporate Governance” and is incorporated herein by reference. The Proxy Statement or Form 10-K/A 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 or Form 10-K/A under the heading “Ratification of Appointment of Independent Registered Public Accounting Firm” and is incorporated herein by reference. The Proxy Statement or Form 10-K/A 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 on Form 10-K:

(1) Financial Statements:

Report of Independent Registered Public Accounting Firm	F-2
Consolidated Balance Sheets as of December 31, 2018 and 2017	F-3
Consolidated Statements of Operations for the Years Ended December 31, 2018, 2017 and 2016	F-4
Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2018, 2017 and 2016	F-5
Consolidated Statements of Changes in Stockholders' Equity for the Years Ended December 31, 2018 and 2017	F-6
Consolidated Statements of Cash Flows for the Years Ended December 31, 2018, 2017 and 2016	F-7
Notes to Consolidated Financial Statements	F-8

(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)
10.1#	2004 Stock Option and Incentive Plan (7)
10.2#	Rocket Pharmaceuticals, Inc. Second Amended and Restated 2014 Stock Option and Incentive Plan (8)
10.3#	Form of Incentive Stock Option Agreement (Employees) (9)
10.4#	Form of Non-Qualified Stock Option Agreement (Employees) (9)
10.5#	Form of Non-Qualified Stock Option Agreement (Non-Employee Directors) (9)
10.6#	Form of Non-Qualified Stock Option Agreement (Consultants) (9)
10.7#	Rocket Pharmaceuticals, Ltd. 2015 Share Option Plan (10)
10.8#	Letter Agreement, dated as of July 28, 2014, by and between the Registrant and David P. Southwell (7)
10.9#	Amendment to Offer Letter, effective as of September 1, 2017, by and between Inotek and David Southwell (11)
10.10#	Letter Agreement, dated as of May 2, 2007, by and between the Registrant and Dr. Rudolf A. Baumgartner, M.D., as amended and currently in effect (7)
10.11#	Amendment to Offer Letter, effective as of August 7, 2017, by and between Inotek and Rudolf A. Baumgartner, MD (12)
10.12#	Amendment to Offer Letter, effective as of September 12, 2017, by and between Inotek and Rudolf A. Baumgartner, MD (1)
10.13#	Letter Agreement, dated as of August 28, 2014, by and between Inotek Pharmaceuticals Corporation and Dale Ritter (7)
10.14#	Amendment to Offer Letter, effective as of September 1, 2017, by and between Inotek and Dale Ritter (11)
10.15#	Letter Agreement, dated as of January 4, 2018, by and between Inotek Pharmaceuticals Corporation and Dale Ritter (10)
10.16#	Rocket Pharmaceuticals, Inc. Amended and Restated 2014 Employee Stock Purchase Plan (10)
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
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 (13)
10.21	First Amendment to Lease, dated as of February 24, 2016, by and between Inotek Pharmaceuticals Corporation and 91 Hartwell Avenue Trust (14)
10.22	Lease Agreement, dated as of March 31, 2016, by and between Rocket Pharmaceuticals, Ltd. and ARE-East River Science Park, LLC. (10)
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. (9)

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 (9)
10.25†	Lease Agreement, dated as of August 14, 2018, by and between Rocket Pharmaceuticals, Inc. and Cedar Brook 12 Corporate Center, L.P. (15)
10.26*	License Agreement, dated as of November 19, 2018, by and between Rocket Pharmaceuticals, Ltd. and REGENXBIO Inc.**
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)
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.

** Portions of this exhibit have been omitted and filed separately with the SEC pursuant to a request for confidential treatment.

- (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 registration statement on Form S-1 (333-199859), filed with the SEC on November 5, 2014, as amended, and incorporated herein by reference.
- (8) 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.
- (9) 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.
- (10) 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.
- (11) 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.

- (12) 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.
- (13) 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.
- (14) 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.
- (15) Filed as an Exhibit to the Company's quarterly report on Form 10-Q (001-36829), filed with the SEC on November 9, 2018, as amended, 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 8, 2019.

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

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

Name	Title	Date
<u>/s/ Gaurav Shah, MD</u> Gaurav Shah, MD	President, Chief Executive Officer and Director <i>(Principal Executive Officer)</i>	March 8, 2019
<u>/s/ John Militello</u> John Militello	Controller <i>(Principal Financial and Accounting Officer)</i>	March 8, 2019
<u>/s/ Carsten Boess</u> Carsten Boess	Director	March 8, 2019
<u>/s/ Pedro Granadillo</u> Pedro Granadillo	Director	March 8, 2019
<u>/s/ Gotham Makker, MD</u> Gotham Makker, MD	Director	March 8, 2019
<u>/s/ David P. Southwell</u> David P. Southwell	Director	March 8, 2019
<u>/s/ Roderick Wong, MD</u> Roderick Wong, MD	Director	March 8, 2019
<u>/s/ Naveen Yalamanchi, MD</u> Naveen Yalamanchi, MD	Director	March 8, 2019

Rocket Pharmaceuticals, Inc.
Index to Consolidated Financial Statements
Contents

Report of Independent Registered Public Accounting Firm	F-2
Consolidated Balance Sheets as of December 31, 2018 and 2017	F-3
Consolidated Statements of Operations for the Years Ended December 31, 2018, 2017 and 2016	F-4
Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2018, 2017 and 2016	F-5
Consolidated Statements of Changes in Stockholders' Equity for the Years Ended December 31, 2018, 2017 and 2016	F-6
Consolidated Statements of Cash Flows for the Years Ended December 31, 2018, 2017 and 2016	F-7
Notes to Consolidated Financial Statements	F-8

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors 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, 2018 and 2017, and the related consolidated statements of operations, comprehensive loss, stockholders’ equity, and cash flows for each of the years in the three-year period ended December 31, 2018, 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, 2018 and 2017, and the consolidated results of their operations and their cash flows for each of the years in the three-year period ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s financial statements based on our 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 8, 2019

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

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 111,355	\$ 18,142
Investments	94,375	-
Prepaid expenses and other assets	3,358	813
Total current assets	209,088	18,955
Property and equipment, net	2,027	985
Goodwill	30,815	-
Restricted cash	1,436	207
Deposits	545	-
Investments	7,402	-
Total assets	\$ 251,313	\$ 20,147
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 15,372	\$ 4,521
Total current liabilities	15,372	4,521
Convertible notes, net of unamortized discount	41,447	-
Deferred rent and lease obligations	457	107
Total liabilities	57,276	4,628
Commitments and contingencies (Note 13)		
Shareholders' equity:		
Preferred shares, \$0.01 par value, authorized 1,000,000 shares:		
Series A convertible preferred shares; 300,000 shares designated as Series A; 0 and 128,738 shares issued and outstanding at December 31, 2018 and 2017, respectively	-	16,060
Series B convertible preferred shares; 300,000 shares designated as Series B; 0 and 126,909 shares issued and outstanding at December 31, 2018 and 2017, respectively	-	25,406
Common stock, \$0.01 par value, 120,000,000 shares authorized; 45,194,736 and 6,795,627 shares issued and outstanding at December 31, 2018 and 2017, respectively	452	68
Treasury stock, at cost, 50,000 and 0 common shares at December 31, 2018 and 2017, respectively	(668)	-
Additional paid-in capital	300,253	5,340
Accumulated other comprehensive loss	(127)	-
Accumulated deficit	(105,873)	(31,355)
Total shareholders' equity	194,037	15,519
Total liabilities and shareholders' equity	\$ 251,313	\$ 20,147

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,		
	2018	2017	2016
Revenue	\$ -	\$ -	\$ -
Operating expenses:			
Research and development	53,270	14,917	5,994
General and administrative	17,886	4,855	1,580
Total operating expenses	<u>71,156</u>	<u>19,772</u>	<u>7,574</u>
Loss from operations	(71,156)	(19,772)	(7,574)
Research and development incentives	186	192	-
Interest expense	(6,039)	-	-
Interest and other income net	1,690	2	1
Accretion of discount on investments	801	-	-
Net loss	<u>\$ (74,518)</u>	<u>\$ (19,578)</u>	<u>\$ (7,573)</u>
Net loss per share attributable to common shareholders - basic and diluted	<u>\$ (1.89)</u>	<u>\$ (2.88)</u>	<u>\$ (1.11)</u>
Weighted-average common shares outstanding - basic and diluted	<u>39,377,666</u>	<u>6,795,627</u>	<u>6,833,718</u>

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,		
	<u>2018</u>	<u>2017</u>	<u>2016</u>
Net loss	\$ (74,518)	\$ (19,578)	\$ (7,573)
Other comprehensive loss			
Net unrealized loss on investments	(127)	-	-
Total comprehensive loss	<u>\$ (74,645)</u>	<u>\$ (19,578)</u>	<u>\$ (7,573)</u>

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 Loss	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount					
Balance at December 31, 2015	127,698	\$ 15,930	-	\$ -	6,795,627	\$ 68	-	\$ 3,746	\$ -	(4,204)	\$ 15,540
Issuance of Series A convertible preferred shares	1,040	130	-	-	-	-	-	-	-	-	130
Share-based compensation	-	-	-	-	-	-	-	274	-	-	274
Net loss	-	-	-	-	-	-	-	-	-	(7,573)	(7,573)
Balance at December 31, 2016	128,738	\$ 16,060	-	\$ -	6,795,627	\$ 68	-	\$ 4,020	\$ -	(11,777)	\$ 8,371
Issuance of Series B convertible preferred shares	-	-	126,909	25,406	-	-	-	-	-	-	25,406
Share-based compensation	-	-	-	-	-	-	-	1,320	-	-	1,320
Net loss	-	-	-	-	-	-	-	-	-	(19,578)	(19,578)
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
Share repurchase	-	-	-	-	-	-	(668)	-	-	-	(668)
Unrealized loss on investments	-	-	-	-	-	-	-	-	(127)	-	(127)
Share-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

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,		
	2018	2017	2016
Operating Activities:			
Net loss	\$ (74,518)	\$ (19,578)	(7,573)
Adjustments to reconcile net loss to net cash used in operating activities:			
Accretion of discount on convertible notes	3,059	-	-
Increase in lease liability	(161)	-	-
Series A preferred shares issued for services	-	-	100
Depreciation expense	330	204	67
Share-based compensation expense	13,601	1,320	274
Deferred rent	-	-	107
Loss on disposal of property and equipment	317	-	-
Accretion of discount on investments	(801)	-	-
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	(1,505)	(720)	78
Accounts payable and accrued expenses	5,890	2,812	1,444
Net cash used in operating activities	<u>(53,788)</u>	<u>(15,962)</u>	<u>(5,503)</u>
Investing activities:			
Cash acquired in connection with the Reverse Merger	76,348	-	-
Purchases of investments	(141,087)	-	-
Proceeds from maturities of investments	61,276	-	-
Proceeds from sale of property and equipment	20	-	-
Purchases of property and equipment	(1,453)	(760)	(335)
Payment of security deposit	(376)	-	-
Net cash used in investing activities	<u>(5,272)</u>	<u>(760)</u>	<u>(335)</u>
Financing activities:			
Proceeds from exercise of options	148	-	-
Proceeds from issuance of common stock, net of issuance costs	154,022	-	-
Proceeds from issuance of Series A convertible preferred shares net	-	-	30
Proceeds from issuance of Series B convertible preferred shares net	-	25,406	-
Payments from related party payable	-	-	(14)
Payment to acquire treasury stock	(668)	-	-
Net cash provided by financing activities	<u>153,502</u>	<u>25,406</u>	<u>16</u>
Net change in cash, cash equivalents and restricted cash	94,442	8,684	(5,822)
Cash, cash equivalents and restricted cash at beginning of year	18,349	9,665	15,487
Cash, cash equivalents and restricted cash at end of year	<u>\$ 112,791</u>	<u>\$ 18,349</u>	<u>\$ 9,665</u>
Supplemental disclosure of non-cash financing and investing activities:			
Conversion of convertible preferred stock into common stock	\$ 41,466	\$ -	\$ -
Unrealized loss on investments	\$ 127	\$ -	\$ -
Supplemental cash flow information:			
Cash paid for interest	\$ 4,485	\$ -	\$ -
Cash paid for income taxes	\$ 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, Merger and Basis of Presentation

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 and Leukocyte Adhesion Deficiency-I (“LAD-I”), a genetic disorder that causes the immune system to malfunction. FA has been in clinical stage testing in the EU since 2016, and in the U.S., Rocket received investigational new drug (“IND”) clearance for both FA and LAD-I in late 2018. Two additional pre-clinical stage LVV programs include Pyruvate Kinase Deficiency (“PKD”), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia; and 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 an adeno-associated virus (“AAV”), program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. An IND filing was cleared in Danon disease in early 2019, which will permit the commencement of human clinical studies following approval. 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.

Reverse Merger and Exchange Ratio

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.

As a result of the Reverse Merger, each outstanding share of Rocket Ltd share capital (including shares of Rocket Ltd share capital to be issued upon exercise of outstanding share options) automatically converted into the right to receive approximately 76.185 shares of Inotek’s common stock, par value \$0.01 per share (the “Exchange Ratio”). Following the closing of the Reverse Merger, holders of Inotek’s common stock immediately prior to the Reverse Merger owned approximately 18.643% on a fully diluted basis, and holders of Rocket Ltd common stock immediately prior to the Reverse Merger owned approximately 81.357% on a fully diluted basis, of Inotek’s common stock.

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.

Immediately following the Reverse Merger, the combined company changed its name from “Inotek Pharmaceuticals Corporation” to “Rocket Pharmaceuticals, Inc.” The combined company following the Reverse Merger may be referred to herein as “the combined company,” “Rocket,” or the “Company.” The Company’s common stock remained listed on the Nasdaq Stock Market, with trading having commenced on a post-split basis (giving effect to the Reverse Stock Split described below) and under the new name as of January 5, 2018. The trading symbol also changed on that date from “ITEK” to “RCKT.”

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 drug 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 \$105,873 as of December 31, 2018. As of December 31, 2018, the Company has \$213,132 of cash, cash equivalents and investments. Rocket expects such resources would be sufficient to fund its operating expenses and capital expenditure requirements into the second half of 2020.

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 and share-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) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's Corporate Credit Card. As of December 31, 2018, and 2017, restricted cash was \$1,436 and \$207, respectively. Cash, cash equivalents and restricted cash consist of the following:

	December 31, 2018	December 31, 2017
Cash and cash equivalents	\$ 111,355	\$ 18,142
Restricted cash	1,436	207
	<u>\$ 112,791</u>	<u>\$ 18,349</u>

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 primarily consist of U.S. Treasury securities, U.S. government agency securities and certificates of deposit, and potentially subject the Company to concentrations of credit risk. 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. 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 shareholders’ 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, 2018, 2017 and 2016. There was \$127 and \$0 of net unrealized losses on investments for the years ended December 31, 2018 and 2017, respectively.

The Company reviews investments for other-than-temporary impairment whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment’s carrying amount is not recoverable within a reasonable period of time. Other-than-temporary impairments of investments are recognized in the consolidated statements of operations if the Company has experienced a credit loss, has the intent to sell the investment, or if it is more likely than not that the Company will be required to sell the investment before recovery of the amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with the Company’s investment policy, the severity and the duration of the impairment and changes in value subsequent to the end of the period. The weighted average maturity of the Company’s investment portfolio is 5.5 months and the average duration of the Company’s long-term investments is 14 months.

Investments at December 31, 2018 consist of the following:

	<u>Cost Basis</u>	<u>Unrealized Losses</u>	<u>Fair Value</u>
United States Treasury securities	101,904	(127)	101,777
	<u>\$ 101,904</u>	<u>\$ (127)</u>	<u>\$ 101,777</u>

The Company evaluated its securities for other-than-temporary impairment and determined that no such impairment existed at December 31, 2018.

Goodwill

Business combinations are accounted for under the acquisition method (see Note 4). 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. As amended, 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. Entities should apply for the amendment prospectively. The Company early adopted this guidance as of January 1, 2018. The Company performed the qualitative assessment of the 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 year ended December 31, 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 five years. 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.

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 conducts 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, no impairment of long-lived assets was noted as of December 31, 2018, 2017 and 2016.

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, prepaid expenses and other assets, 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, 2018 was \$43,182. The Company's assets and liabilities measured at fair value on a recurring basis include its short and long term investments.

Deferred Rent and Lease Liability

The Company recognizes rent expense on a straight-line basis, after considering the effect of rent escalation provisions resulting in a level rent expense recognized over the lease term. For the lease liability, the Company reduces the rent expense on a straight-line basis over the remaining life of the lease.

Research and Development

Research and development costs, which include salaries and staff costs, license costs, regulatory and scientific consulting fees, as well as contract research, and share-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, research and development costs are expensed as incurred. While certain of the Company's research and development costs may have future benefits, the policy of expensing all research and development 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, 2018, 2017 and 2016 are denominated in Euros. Gains and losses on foreign currency transactions are not significant for the years ended December 31, 2018, 2017 and 2016.

Treasury Stock

The Company records treasury stock at cost. Treasury stock consists of 50,000 shares that were settled in December 2018.

Share-Based Compensation

The Company measures the cost 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 share-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.

The Company measures the compensation expense of share options and other share-based awards granted to employees, nonemployees and directors using the grant date fair value of the award and recognizes compensation expense as determined by the Black-Scholes Option pricing model on a straight-line basis over their requisite service period, which is generally the vesting period of the respective award.

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 share-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, 2018, 2017 and 2016, the Company recorded research and development incentive income of \$186, \$192 and \$0, 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 gross 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, 2018 and 2017.

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.

On December 22, 2017, the Tax Cut and Jobs Act (the "Act"), was signed into law by the President of the United States. The Act includes a number of provisions, including the lowering of the U.S. corporate tax rate from 34 percent to 21 percent, effective January 1, 2018 and the establishment of a territorial-style system for taxing foreign-source income of domestic multinational corporations.

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 and convertible preferred shares are considered potential dilutive common shares. In connection with the Reverse Merger, all of the convertible preferred shares were converted into common stock. This conversion was in accordance with the original terms.

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

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*, as amended, which supersedes the current leasing guidance and upon adoption, will require lessees to recognize right-of-use assets and lease liabilities on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. The new standard is effective for the Company for the annual period beginning after December 15, 2018, and can be early adopted. Upon the adoption of the guidance, operating leases are capitalized on the balance at the present value of lease payments. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 must be calculated using the applicable incremental borrowing rate at the date of adoption. The Company completed its calculations of the assets and liabilities to be recorded and required additional disclosures upon the adoption of ASU 2016-02.

The FASB issued ASU 2018-11 in July 2018, which includes certain amendments to ASU 2016-02 intended to provide relief in implementing the new standard. Among these amendments is the option to not restate comparative periods presented in the financial statements. The Company intends to elect this new transition approach, using a cumulative-effect adjustment on the effective date of the standard, with comparative periods presented in accordance with the previous guidance in ASC 840.

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 expects it will make 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.

In preparation for adoption of the standard, the Company has implemented internal controls to enable the preparation of financial information including the assessment of the impact of the standard. The adoption of the new standard is expected to result in the recognition of additional lease liabilities ranging from \$3.0 million to \$3.7 million, and right-of-use assets ranging from \$2.6 million to \$3.2 million as of January 1, 2019 related to the Company's operating leases.

In January 2017, the FASB issued ASU 2017-01, “Business Combinations (Topic 805): Clarifying the Definition of a Business” (“ASU 2017-01”). ASU 2017-01 provides that when substantially all of the fair value of the assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the set is not a business. ASU 2017-01 is effective to annual periods beginning after December 31, 2018 and interim periods within annual periods beginning after December 31, 2019. Adoption of ASU 2017-01 may impact the Company’s accounting for future acquisitions.

4. Acquisition Accounting

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	<u>\$ 86,060</u>

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 its 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	<u>\$ 86,060</u>

The goodwill of \$30,815 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). The Company incurred and expensed acquisition costs of \$147 for the year ended December 31, 2018.

The following supplemental unaudited pro forma information presents the Company’s financial results as if the acquisition of Inotek had occurred on January 1, 2017:

	Year Ended December 31,	
	2018	2017
Revenue	\$ -	\$ -
Net loss	(78,248)	(35,760)

The above unaudited pro forma information was determined based on the historical US GAAP results of the Company and Inotek. The unaudited pro forma consolidated results are not necessarily indicative of what the Company’s consolidated results of operations actually would have been if the acquisition was completed on January 1, 2017. The unaudited pro forma consolidated net loss includes pro forma adjustments primarily relating to the following non-recurring items directly attributable to the business combination:

- (1) Elimination of \$4,512 of transaction costs for both the Company and Inotek from the year ended December 31, 2018;
- (2) Elimination of \$3,459 of stock-based compensation expense related to the acceleration of vesting and modification of certain previously unvested Inotek awards in connection with the Reverse Merger from the year ended December 31, 2018;
- (3) Elimination of \$1,622 of expense related to severance and stay bonuses from the year ended December 31, 2018;
- (4) To adjust interest expense incurred in connection with the 2021 Convertible Notes assumed in connection with the Reverse Merger based on the fair value of the 2021 Convertible Notes on the date of the Reverse Merger, as if it occurred on January 1, 2017;

- (5) To adjust depreciation expense associated with property and equipment acquired in connection with the Reverse Merger based on the fair value of the property and equipment on the date of the Reverse Merger, as if it occurred on January 1, 2017; and
- (6) To adjust expense associated with operating lease obligations assumed in connection with the Merger based on the fair value of the leases on the date of the Merger, as if it occurred on January 1, 2017.

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, 2018 Using:			Total
	Level 1	Level 2	Level 3	
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
	<u>\$ 132,329</u>	<u>\$ -</u>	<u>\$ -</u>	<u>\$ 132,329</u>

The Company classifies its money market mutual funds and United States 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.

6. Property and Equipment

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

	December 31, 2018	December 31, 2017
Laboratory equipment	\$ 1,556	\$ 1,042
Computer equipment	179	98
Furniture and fixtures	273	115
Construction in progress	469	—
Leasehold improvements	29	—
	<u>2,506</u>	<u>1,255</u>
Less: accumulated depreciation	<u>(479)</u>	<u>(270)</u>
	<u>\$ 2,027</u>	<u>\$ 985</u>

During the years ended December 31, 2018, 2017 and 2016, the Company recognized \$330, \$204 and \$67 of depreciation expense, respectively.

7. Accounts Payable and Accrued Expenses

At December 31, 2018 and 2017, the Company's accounts payable and accrued expenses consisted of the following:

	December 31, 2018	December 31, 2017
Bonus	\$ 1,774	\$ 703
Research and development	10,414	3,273
Severance and benefits	7	138
Professional fees	690	382
Government grant payable	534	—
Accrued interest	1,241	—
Accrued vacation	123	—
Other	589	25
	<u>\$ 15,372</u>	<u>\$ 4,521</u>

8. 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 \$52,000, 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.

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 the fair value of the Additional Interest feature to be insignificant upon issuance, at the time of the Reverse Merger, and as of December 31, 2018.

The Company recorded the 2021 Convertible Notes at their fair value of \$38,388 on January 4, 2018, the date of the acquisition. The difference between the fair value of the 2021 Convertible Notes and the principal value represents a discount on the notes that is being amortized to interest expense over the remaining term using the effective interest method. As of December 31, 2018, 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 as of December 31, 2018:

Principal amount	\$ 52,000
Discount	(10,553)
Carrying value as of December 31, 2018	<u>\$ 41,447</u>

Accretion of the 2021 Convertible Notes discount was \$3,059 for the year ended December 31, 2018.

9. Shareholders' Equity

Preferred Shares

On January 4, 2018, immediately prior to and in connection with the closing of the Reverse Merger, and in accordance with the original terms of the convertible preferred shares, all of the outstanding convertible preferred shares of Rocket Ltd were converted into an aggregate of 19,475,788 shares of common stock.

Exchange Ratio

On January 4, 2018, in connection with the Reverse Merger, Rocket's historical share and per share information has been adjusted in the consolidated financial statements presented to give effect to the Exchange Ratio.

Common Shares

At the time of the Reverse Merger, Rocket Ltd's outstanding shares of common stock were 26,281,396 which includes 19,475,788 issued upon the conversion of Rocket Ltd's convertible preferred stock.

On January 24, 2018, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Cowen and Company, LLC and Evercore Group L.L.C., as representatives (the "Representatives") of the several underwriters (collectively with the Representatives, the "Underwriters"), pursuant to which the Company sold 6,325,000 shares of common stock (the "Shares"), which includes 825,000 shares that were sold pursuant to an option granted to the Underwriters (the "Offering"). The Shares were sold in the Offering at a public offering price of \$13.25 per share in which the Company received gross proceeds of \$83,806 net of \$5,288 of offering costs, commission and legal and other expenses for net proceeds from the Offering of \$78,518, after deducting the underwriting discounts and commissions and legal and accounting costs.

On November 30, 2018, the Company announced the closing of its previously announced underwritten 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 offering were approximately \$63,339, net of \$4,035 of offering costs, commissions, legal and other expenses for a net proceeds from the offering of \$59,304. BofA Merrill Lynch, Cowen and Company, LLC and Evercore ISI acted as joint book-running managers for the offering. Oppenheimer & Co. acted as lead manager and Ladenburg Thalmann acted as co-manager.

In addition to the shares sold in the public offering, on November 30, 2018 Rocket closed a concurrent sale of 967,742 shares of common stock at a price of \$15.50 per share, for gross proceeds of \$15,000, net of offering costs of \$200 for net proceeds of \$14,800, 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 December 27 and 28, 2018, the Company repurchased 100,000 shares of its common stock for aggregate consideration of approximately \$1,400. 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 by the Company settled on December 31, 2018 and the remaining 50,000 shares repurchased at an average price of \$14.50 settled on January 2, 2019. As of December 31, 2018, at an average price of \$13.36, the Company recorded a prepaid expense of \$726 related to the 50,000 shares that settled on January 2, 2019 and recorded treasury stock of \$668 relating to the 50,000 shares that settled as of December 31, 2018. These shares were subsequently retired in January 2019.

Immediately following the stock repurchases (the “Repurchases”), the Company sold 100,000 shares of common stock (the “Shares”) for \$1,396 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.

10. Share-Based Awards

2015 Share Option Plan

The Rocket Ltd 2015 Share Option Plan provides for the Company to grant incentive stock options or nonqualified stock options for the purchase of common shares to employees, members of the board of directors and consultants. The 2015 Share Option Plan is administered by an administrative committee appointed by the board of directors or, in the absence of such appointment, the entire board of directors. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or their committee if so delegated, except that the exercise price per share of share options may not be less than 100% of the fair market value of the share of common shares on the date of grant (or 110% of the fair market value in the case of an employee who owns shares representing more than 10% of the voting power of all classes of shares for the Company) and the term of share options may not be greater than ten years (or five years in the case of an employee who owns shares representing more than 10% of the voting power of all classes of shares for the Company). The Company generally grants share-based awards with service conditions only (“service-based” awards).

As required by the 2015 Share Option Plan, the exercise price for share options granted was not to be less than the fair value of common shares as determined by the Company as of the date of grant. The Company valued its common shares by taking into consideration its most recently available valuation of common shares performed by management and the board of directors as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant.

The total number of shares that may be issued under the 2015 Share Option Plan was 9,904,050 shares; however, the 2,944,702 shares that remained available under the 2015 Share Option Plan were added to the share reserve of the 2014 Plan in connection with the Reverse Merger.

By virtue of the terms of the Merger Agreement, each stock option outstanding under the Rocket Ltd 2015 Share Option Plan immediately prior to the consummation of the Reverse Merger was automatically converted into a stock option exercisable for a number of shares of the Company’s common stock calculated based on the exchange ratio and the exercise price per share of such outstanding stock option.

Pursuant to the Merger Agreement, the Company sponsored Inotek’s equity compensation plans: the Amended and Restated 2014 Stock Option and Incentive Plan (the “2014 Plan”), the 2004 Stock Option and Incentive Plan (the “2004 Plan”), and the 2014 Employee Stock Purchase Plan (“ESPP”) and assumed all stock options and restricted stock units (“RSUs”) outstanding under each of the plans immediately prior to the effective time of the Reverse Merger.

Second Amended and Restated 2014 Stock Option and Incentive Plan

In March 2018, Rocket’s board of directors approved the Second Amended and Restated 2014 Stock Option and Incentive Plan (the “Revised 2014 Plan”) which was subsequently approved by the Company’s shareholders at the Annual Meeting held on June 25, 2018. The Revised 2014 Plan contains the following material features and changes from the 2014 Plan:

- Provides for an aggregate maximum number of shares of common stock initially authorized for issuance of 4,294,830 shares. On January 1, 2019, and each January 1 thereafter for the term of the Revised 2014 Plan, the number of shares reserved and available under the Revised 2014 Plan will automatically increase by 4% of the number of shares of our common stock issued and outstanding on the immediately preceding December 31;
- Increase the number of shares of stock underlying stock options or stock appreciation rights that may be granted to any one individual in any single calendar year to 1,000,000 shares of common stock, and an increase in the number of shares of stock that may be issued in the form of incentive stock options;
- Eliminates certain provisions relating to awards of “performance-based compensation”; and
- Expires on June 25, 2028.

2004 Stock Option and Incentive Plan

In July 2004, Inotek’s board of directors adopted the 2004 Plan for the issuance of incentive stock options, restricted stock, and other equity awards, all for common stock, as determined by the board of directors to employees, officers, directors, consultants, and advisors of Inotek and its subsidiaries. Only stock options were granted under the 2004 Plan. The 2004 Plan expired in February 2014 but remains effective for all outstanding options. All of the stock options granted under the 2004 Plan were fully vested at the time of the Reverse Merger.

Vesting of all unvested Inotek option awards issued and outstanding was accelerated at the effective time of the Reverse Merger, and all such option awards issued and outstanding at the time of the Reverse Merger, aggregating to 523,456, remained issued and outstanding. For accounting purposes, since the acceleration of vesting was negotiated in contemplation of the Reverse Merger, the remaining unrecognized compensation expense associated with the original grant date fair value of the options of \$2,997 was recognized in the Company’s consolidated statement of operations for the year ended December 31, 2018. In addition, the exercise period for all Inotek options outstanding at the effective time of the Reverse Merger was extended beyond the respective periods provided in the original awards. The Company recorded \$462 in connection with the extension of the exercise periods in the consolidated statement of operations for the year ended December 31, 2018, equal to the difference in the fair value of the options immediately prior to and immediately following the modification of the exercise period.

Share Option Valuation

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

	Years Ended December 31,		
	2018	2017	2016
Risk-free interest rate	2.64%	2.06%	1.65%
Expected term (in years)	5.85	6.00	6.00
Expected volatility	87.7%	91.3%	96.10%
Expected dividend yield	0.00%	0.00%	0.00%
Exercise price	\$ 17.82	\$ 2.97	\$ 1.21
Fair value of common stock	\$ 17.98	\$ 2.63	\$ 0.98

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

	Years Ended December 31,		
	2018	2017	2016
Risk-free interest rate	2.85%	2.39%	2.39%
Expected term (in years)	9.75	6.70	5.87
Expected volatility	82.19%	90.62%	106.15%
Expected dividend yield	0.00%	0.00%	0.00%
Exercise price	\$ 18.75	\$ 1.21	\$ 1.21
Fair value of common stock	\$ 19.63	\$ 1.08	\$ 1.11

The Company recognizes stock-based compensation expense for at least the portion of awards that have vested.

The following table summarizes stock option activity for the years ended December 31, 2018 and 2017, under the Revised 2014 Plan:

		<u>Number of Shares</u>	<u>Weighted Average Exercise Price</u>	<u>Weighted Average Contractual Term (Years)</u>
Outstanding as of December 31, 2015	*	5,332,950	\$ 0.69	9.90
Granted		1,005,871	1.21	7.23
Forfeited		(146,656)	1.21	9.13
Outstanding as of December 31, 2016	*	6,192,164	\$ 0.79	9.00
Granted		812,894	2.98	5.50
Forfeited		(45,711)	1.21	8.88
Outstanding as of December 31, 2017	*	6,959,347	\$ 1.06	8.17
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		<u>8,615,997</u>	\$ 4.48	7.51
Options vested and exercisable as of December 31, 2018		6,466,265	\$ 1.22	6.52

* Affected by Exchange Ratio

Restricted Stock Units

All unvested Inotek RSU awards issued and outstanding were accelerated at the effective time of the Reverse Merger. For accounting purposes, since the acceleration of vesting upon change of control was included in the original terms of the award, the remaining unrecognized compensation expense associated with the original grant date fair value of the RSU awards was recognized as a pre-merger expense of Inotek.

The following table summarizes the RSU activity for the year ended December 31, 2018 under the Revised 2014 Plan:

	<u>Number of Shares</u>
Outstanding as of December 31, 2017	-
Assumed as part of merger with Inotek	271,719
Settled	(271,719)
Outstanding as of December 31, 2018	<u>-</u>

As of December 31, 2018, none of the RSU's remain unsettled.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock. The intrinsic value of options exercised and exercisable as of December 31, 2018 was \$6 and \$88.

The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2018, 2017 and 2016 was \$13.21, \$2.23, and \$74.15, respectively.

The total fair value of options vested during the years ended December 31, 2018, 2017 and 2016 was \$91,334, \$502, and \$845 respectively.

Employee Stock Purchase Plan

In November 2014, Inotek's board of directors adopted and the stockholders approved the ESPP. The ESPP provides that the number of shares reserved and available for issuance under the ESPP shall be cumulatively increased each January 1, beginning on January 1, 2016, by the lesser of (i) 150,000 shares of common stock or (ii) the number of shares necessary to set the number of shares of common stock under the ESPP at 1% percent of the outstanding number of shares as of January 1 of the applicable year. However, the board of directors reserves the right to determine that there will be no increase for any year or that any increase will be for a lesser number of shares. On January 1, 2018, 6,562 shares were added to the ESPP. On September 11, 2018, the board of directors determined that there would be no increase in shares reserved and available for issuance under the ESPP on January 1, 2019. As of December 31, 2018, there were 68,256 shares available for issuance under the ESPP. During the year ended December 31, 2018, 0 shares of common stock were purchased and the Company recorded \$0 of stock-based compensation expense pursuant to the ESPP.

Share-Based Compensation

	Year Ended December 31,		
	2018	2017	2016
Research and development	\$ 7,375	\$ 523	\$ 159
General and administrative	6,226	797	115
Total share based compensation expense	\$ 13,601	\$ 1,320	\$ 274

As of December 31, 2018, the Company had an aggregate of \$18,272 of unrecognized share-based compensation cost, which is expected to be recognized over the weighted average period of 2.05 years.

11. Net Loss Per Share

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

	For the Years Ended December 31,		
	2018	2017	2016
Numerator:			
Net loss attributable to common shareholders	\$ (74,518)	\$ (19,578)	\$ (7,573)
Denominator:			
Weighted-average common shares outstanding - basic and diluted	39,377,666	6,795,627	6,833,718
Net loss per share attributable to common shareholders - basic and diluted	\$ (1.89)	\$ (2.88)	\$ (1.11)

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

	For the Years Ended December 31,		
	2018	2017	2016
Shares issuable upon conversion of the 2021 Convertible Notes	1,620,948	-	-
Warrants exercisable for common shares	14,102	-	-
Options to purchase common shares	8,615,997	6,756,695	6,192,164
Redeemable Series A convertible preferred shares (as converted to common shares)	-	9,807,564	9,807,905
Redeemable Series A convertible preferred shares (as converted to common shares)	-	9,668,224	-
	10,251,047	26,232,483	16,000,069

The Company has warrants of 14,102 outstanding as of December 31, 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

Net loss by jurisdiction consists of the following:

	For the years ended December 31,		
	2018	2017	2016
Domestic	\$ 74,518	\$ -	\$ -
Foreign	-	19,578	7,573
Total	\$ 74,518	\$ 19,578	\$ 7,573

No provision for federal or state income taxes was recorded during the years ended December 31, 2018, 2017, and 2016, 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,		
	2018	2017	2016
U.S. federal tax at statutory rate	21.0%	34.0%	34.0%
Foreign source income not subject to tax	0.0%	-34.0%	-34.0%
State income taxes	15.8%	0.0%	0.0%
New York City tax	-	6.5%	6.3%
Other	-1.6%	-	-
Section 382 limitation	-38.7%	-	-
Valuation allowance	3.5%	-6.5%	-6.3%
Effective tax rate	0%	0%	0%

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

	For the years ended December 31,		
	2018	2017	2016
Deferred income tax assets (liabilities)			
Net operating losses ("NOL") and credit carryforwards	\$ 28,400	\$ 1,620	\$ 458
Capitalized research and development costs	17,098	-	-
Other	1,385	136	25
Stock compensation	4,563	-	-
Debt discount	(3,652)	-	-
Valuation allowance	(47,794)	(1,756)	(483)
Net deferred income tax asset	\$ -	\$ -	\$ -

As of December 31, 2018, the Company had federal and state net operating loss carryforwards of approximately \$96,400 and \$124,500, respectively, which begin to expire in 2025. Additionally \$62,300 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, 2018 and 2017. 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, 2018, 2017 and 2016, the valuation allowance increased by \$46,016 and \$1,273 respectively. Realization of deferred tax assets is dependent upon the generation of future taxable income.

During 2018, the Company recorded tax charges for the impact of the Tax Act effects using the currently available information and technical guidance on the interpretations of the Tax Act. As permitted by SEC Staff Accounting Bulletin 118, *Income Tax Accounting Implications of the Tax Cuts and Jobs Act*, the Company recorded provisional estimates and have subsequently finalized our accounting analysis based on the guidance, interpretations, and data available as of December 31, 2018. Adjustments made in the fourth quarter of 2018 upon finalization of our accounting analysis were not material to our Consolidated Financial Statements.

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 \$1,700 annual limitation on our ability to utilize our pre-merger NOL's and R&D Credits. Due to this limitation, approximately \$93,000 of the \$127,100 pre-merger Federal NOL will expire unutilized as the cumulative limitation amount over a 20 year carryforward period is \$34,100. Additionally, \$4,900 of Federal R&D Credits will expire unutilized. As a result, the Company has reduced its deferred tax assets related to the Federal NOL and Federal R&D Credits which is offset by the corresponding 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, 2018 the Company has not recorded any uncertain tax positions in our 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, 2018 and 2017, no accrued interest or penalties are included on the related tax liability line in the consolidated balance sheet.

13. Commitments and Contingencies

Operating Leases

On March 31, 2016, the Company entered into a lease agreement for its office and laboratory space at the Alexandria Center for Life Sciences in New York, New York with a term ending on July 31, 2021 (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. The Company recorded an additional rent expense of \$94 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 \$89 (the "New ARE LOC") relating to the remaining lab space and the existing ARE LOC of \$209 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 with ESRT Empire State Building, L.L.C. 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 \$936 which expires on June 30, 2019 and is renewed automatically for a one year period until its expiration date of September 30, 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 \$936 with a bank as collateral for the Empire LOC which is classified as part of restricted cash in the consolidated balance sheet as of December 31, 2018.

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 May 2018, the Company separated from the last legacy employee of Inotek and abandoned use of the leased space at that time. In connection with the abandonment of the Massachusetts lease, the Company recorded a liability at the present value of the difference between the lease payments and projected sublease income at the cease use date of \$435. The difference between the lease liability recorded at acquisition and the \$435 lease abandonment is a deferred gain which is being recognized to other income over the remaining life of the lease. In addition, as of May 2018, the Company wrote off the remaining furniture and fixtures of \$205. 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.

On August 14, 2018, Rocket entered into a lease for approximately 92,000 rentable square feet in Cranbury, New Jersey, for office space, process development, research activities and potentially manufacturing to support the Company's pipeline (the "NJ Lease Agreement"). The term of the NJ Lease Agreement will commence for 72,000 rentable square feet upon substantial completion of leasehold improvements (the "Commencement Date"), and the remaining 20,000 square feet will commence upon the earlier of the Company's election to commence the lease of such additional space or thirty months from the Commencement Date. The NJ Lease Agreement has a term of fifteen years from the Commencement Date, with an option to renew for two consecutive five-year renewal terms. Estimated rent payments are \$1,200 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 \$26,492 over the 15 year term of the lease. The Company delivered a cash security deposit of \$287 to the landlord in connection with the NJ Lease Agreement which has been reflected in deposits in the consolidated balance sheets.

The total restricted cash balance for the Company's operating leases at December 31, 2018 is \$936. Rent expense was \$807, \$563 and \$358 for the years ended December 31, 2018, 2017 and 2016.

As of December 31, 2018, the remaining aggregate annual commitments pursuant to the leases, as amended, are as follows:

2019	\$	1,372
2020		1,953
2021		1,841
2022		1,739
2023		1,615
Thereafter		20,144
Total	\$	28,664

Securities Litigation

On January 6, 2017, a purported stockholder of Inotek filed a putative class action in the U.S. District Court for the District of Massachusetts, captioned *Whitehead v. Inotek Pharmaceuticals Corporation, et al.*, No. 1:17-cv-10025. An amended complaint was filed on July 10, 2017, and a second amended complaint was filed on September 5, 2017. The second amended complaint alleged violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and SEC Rule 10b-5 against Inotek, David Southwell, and Rudolf Baumgartner based on allegedly false and misleading statements and omissions regarding Inotek's phase 2 and phase 3 clinical trials of *trabodenason*. The lawsuit sought, among other things, unspecified compensatory damages for purchasers of Inotek's common stock between July 23, 2015 and July 10, 2017, as well as interest and attorneys' fees and costs. The second amended complaint was dismissed with prejudice on June 27, 2018. Plaintiffs filed a notice of appeal to the First Circuit Court of Appeals on July 27, 2018, voluntarily dismissed their appeal on August 31, 2018, and the First Circuit entered judgment dismissing the action on September 5, 2018.

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

License 161101 and SRA 161101

On April 20, 2018, the Company entered into Amendment No. 1 to the clinical trial agreement with the Fred Hutchinson Research Cancer Center ("Hutch") for the clinical trial entitled: Gene Therapy for Patients with Fanconi Anemia Complementation Group A. The Company agreed to pay \$108 for additional budgeted amounts for the period from March 2018 through August 2018.

LAD-1 (leukocyte adhesion deficiency-I) Master Research Agreement with CIEMAT

On March 1, 2018, the Company entered into Amendment No. 1 to the LAD-1 Master Research Agreement whereby the Company and Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas ("CIEMAT") agreed to a modification of the original commitment from Rocket of €1,690 down by €361 (approximately \$444).

PKD (pyruvate kinase deficiency) Master Research Agreement with CIEMAT

On September 17, 2018, the Company entered into Amendment No. 1 to the PKD Master Research Agreement whereby the Company and CIEMAT agreed to a modification of the original commitment from Rocket of €4,190 down by €1,007 (approximately \$1,168).

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 Osteopetrosis. 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 Osteopetrosis. 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.

Rocket may terminate this agreement at any time by providing Lund University and Dr. Richter with 90 days' advance notice. The research agreement has an initial term of 24 months. In August 2018, the research and collaboration agreement was renewed for an additional year expiring August 2019.

License Agreement for Danon Disease

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 the Company enters into a sublicense agreement with a sublicensee, the Company will be obligated to pay a portion of any consideration received from such sublicensees in specified circumstances. 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 through its wholly-owned subsidiary Rocket Pharmaceuticals, Ltd., entered into a License Agreement (the "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 AAVvectors 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 4 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 \$7,000 included as research and development expenses. A fee of \$2,000 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 \$13,000 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.

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 \$3,500 for the LCGM expansion upon which 40% or \$1,400 was due upon execution of the agreement and the balance is due upon the achievement of certain milestones. \$1,400 of the \$3,500 milestone payments was paid during the year ended December 31, 2018. As of December 31, 2018, none of the remaining milestones were met with regard to the LCGM. 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 \$1,400 for the LCGM became due and was expensed in January 2019, when the milestone was met.

16. Related Party Transactions

During January 2018, the Company sold certain furniture and fixtures from the Inotek former corporate headquarters to a director of the Company for \$20.

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 \$210 in connection with this consulting agreement.

During April 2018, the Company entered into a consulting agreement with a different member of the Board of Directors for business development consulting services. Payments for the services under the agreement are \$28 per quarter, and the Company may terminate the agreement with 14 days’ notice. The Company incurred expenses of \$110 in connection with this consulting agreement.

17. 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 year ended December 31, 2018, 2017 and 2016, was \$112, \$39 and \$28, respectively.

18. Selected Quarterly Financial Data (unaudited)

The following table contains quarterly financial information for 2018 and 2017. The Company believes that the following information reflects all normal recurring adjustments necessary for a fair presentation of the information for the periods presented. The operating results for any quarter are not necessarily indicative of results for any future period.

	2018			
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
Total revenue	\$ -	\$ -	\$ -	\$ -
Total operating expenses	14,405	14,872	15,333	26,546
Loss from operations	(14,405)	(14,872)	(15,333)	(26,546)
Net loss	(15,343)	(15,767)	(16,089)	(27,319)
Net loss per common share—basic and diluted	\$ (0.42)	\$ (0.40)	\$ (0.40)	\$ (0.66)

	2017			
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
Total revenue	\$ -	\$ -	\$ -	\$ -
Total operating expenses	2,870	3,521	6,725	6,656
Loss from operations	(2,870)	(3,521)	(6,725)	(6,656)
Net loss	(2,679)	(3,521)	(6,723)	(6,655)
Net loss per common share—basic and diluted	\$ (0.39)	\$ (0.49)	\$ (0.99)	\$ (0.98)

FORM OF SEVERANCE AND CHANGE IN CONTROL AGREEMENT

This Severance and Change in Control Agreement (the "Agreement") is made and entered into as of [], by and between [] ("Employee") and Rocket Pharmaceuticals, Inc., a Delaware Corporation (the "Company").

WHEREAS, the Company employs Employee as its []; and

WHEREAS, the Company wishes to provide for certain payments to Employee in the event of a qualifying termination of employment or a change in control of the Company, as defined herein;

NOW, THEREFORE, in consideration of Employee's continuing service to the Company and of the mutual covenants, promises and obligations set forth herein between the parties, the parties agree as follows:

1. At-Will Status.

a. Employee and the Company understand and agree that Employee's employment with the Company is at-will, meaning that the Company or Employee may terminate Employee's employment at any time, with or without reason or notice, and nothing in this Agreement constitutes a promise to employ or to continue to employ Employee for any period of time or is otherwise intended to constitute a contract or guarantee of continued employment; provided, however, that Employee shall be entitled to severance or Change in Control payments (as that phrase is defined below) as set forth in this Agreement.

b. For purposes of this Agreement, "Change in Control" is defined as (i) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (ii) a merger, reorganization or consolidation pursuant to which the holders of the Company's outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or other equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction, (iii) the sale of all of the stock of the Company to an unrelated person, entity or group thereof acting in concert, or (iv) any other transaction in which the owners of the Company's outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

2. Termination for Cause or Without Good Reason. If Employee's employment is terminated by the Company for Cause or by Employee without Good Reason, Employee shall be entitled to receive:

a. any accrued but unpaid base salary and accrued but unused vacation which shall be paid within one (1) month following the Termination Date (as defined below) in accordance with the Company's customary payroll procedures or such earlier date as may be required by applicable law;

- b. any unpaid annual bonus with respect to any completed fiscal year immediately preceding the Termination Date; provided, however, that, if Employee's employment is terminated by the Company for Cause, then any such accrued but unpaid annual bonus shall be forfeited;
- c. reimbursement for unreimbursed business expenses properly incurred by Employee, which shall be subject to and paid in accordance with the Company's expense reimbursement policy; and
- d. such employee benefits, if any, to which Employee may be entitled under the Company's employee benefit plans as of the Termination Date; provided that, in no event shall Employee be entitled to any payments in the nature of severance or termination payments except as specifically provided herein.
- e. Items 2(a) through 2(d) are referred to herein collectively as the "**Accrued Amounts**".
- f. For purposes of this Agreement, "**Cause**" shall mean, as determined by the Board of Directors of the Company (the "Board"), in their discretion exercised in good faith, Employee's dismissal as a result of: (i) any material breach by the Employee of any agreement between the Employee and the Company; (ii) the conviction of, indictment for or plea of nolo contendere by the Employee to a felony or a crime involving moral turpitude; or (iii) any material misconduct or willful and deliberate non-performance (other than by reason of the employee's Disability) by the Employee of the Employee's duties to the Company.
- g. For purposes of this Agreement, "**Good Reason**" shall mean the occurrence of any of the following, in each case during the term of employment without Employee's written consent, which circumstances are not remedied by the Company within thirty (30) days of its receipt of a written notice from the Employee describing the applicable circumstances (which notice must be provided by the Employee within ninety (90) days of the Employee's knowledge of the applicable circumstances), of one or more of the following:
- i. a material, adverse change in the Employee's duties, responsibilities, authority, title or reporting structure;
 - ii. a material reduction in the Employee's base salary or bonus opportunity; or
 - iii. a geographical relocation of the Employee's principal office location by more than fifty (50) miles.

h. Without Cause or for Good Reason. If Employee's employment with the Company is terminated by the Company without Cause or by Employee for Good Reason, Employee shall be entitled to receive the Accrued Amounts. If Employee executes a release of claims in favor of the Company, its affiliates and their respective officers and directors in a form provided by the Company (the "Release"), then Employee will receive, subject to such Release becoming irrevocable, in addition to the Accrued Amounts:

- i. a lump sum payment equal to [nine (9) / twelve (12)] months of Employee's base salary for the year in which the Termination Date occurs, which shall be paid within thirty (30) days following Employee's execution and non-revocation of the Release provided that if the effective date of such Release could span two calendar years depending on the date on which the Employee signs the Release, the payment will not be made until the later calendar year; and
- ii. If Employee timely elects health continuation coverage under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("COBRA"), the Company shall reimburse Employee for the monthly COBRA premium paid by Employee for [him / her]self and her dependents for a period of [nine (9) / twelve (12)] months following the Termination Date.

i. Death or Disability. Employee's employment hereunder shall terminate automatically upon Employee's death, and the Company may terminate Employee's employment on account of Employee's Disability. If Employee's is terminated on account of Employee's death or Disability, Employee (or Employee's estate and/or beneficiaries, as the case may be) shall be entitled to receive the following:

- i. the Accrued Amounts; and
- ii. a pro-rata portion of the annual bonus, if any, that Employee would have earned for the calendar year in which the Termination Date occurs, the determination of such annual bonus to remain in the sole and absolute discretion of the Board.

j. For purposes of this Agreement, Disability shall mean, as determined by management or the Board, in their discretion exercised in good faith, the Employee's inability to engage in the activities required by the Employee's position at the Company by reason of any medically determinable and documented physical or mental impairment which can reasonably be expected to result in death or to last for a continuous period of not less than 24 months..

3. Change in Control Termination.

a. Notwithstanding any other provision contained herein, if Employee's employment hereunder is terminated by Employee for Good Reason or by the Company without Cause (other than on account of Employee's death or Disability), in each case within twelve (12) months following a Change in Control, Employee shall be entitled to receive, subject to Employee's execution and non-revocation of a Release:

- i. the Accrued Amounts and a lump sum amount equal to [eighteen (18) months of the] Employee's annual salary during the fiscal year in which the termination occurs (not including any reduction of such salary leading to Employee's termination with Good Reason) which shall be paid within thirty (30) days following Employee's execution and non-revocation of the Release provided that if the effective date of such Release could span two calendar years depending on the date on which the Employee signs the Release, the payment will not be made until the later calendar year;
- ii. A lump sum amount equal to any annual bonus to which Employee would have been entitled during the fiscal year in which the termination occurred, such annual bonus to be determined in the sole and absolute discretion of the Board and consistent with any bonus awarded to the Company's [] in that same year; and
- iii. If Employee timely elects health continuation coverage under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("**COBRA**"), the Company shall reimburse Employee for the monthly COBRA premium paid by Employee for [him / her]self and [his / her] dependents for a period of [twelve (12) / eighteen (18)] months following the Termination Date.

b. Notwithstanding the foregoing, a Change in Control shall not occur unless such transaction constitutes a change in the ownership of the Company, a change in effective control of the Company, or a change in the ownership of a substantial portion of the Company's assets under Section 409A of the Internal Revenue Code ("Section 409(A)"). Further, notwithstanding anything contained herein, the Termination Date shall not occur until the date on which Employee incurs a "separation from service" within the meaning of Section 409A.

4. Section 280G.

a. If any of the payments or benefits received or to be received by Employee (including, without limitation, any payment or benefits received in connection with a Change in Control or Employee's termination of employment, whether pursuant to the terms of this Agreement or any other plan, arrangement or agreement, or otherwise) (all such payments collectively referred to herein as the "280G Payments") constitute "parachute payments" within the meaning of Section 280G of the Internal Revenue Code and would, but for this Section 4, be subject to the excise tax imposed under Section 4999 of the Code (the "Excise Tax"), then prior to making the 280G Payments, a calculation shall be made comparing (i) the Net Benefit (as defined below) to Employee of the 280G Payments after payment of the Excise Tax to (ii) the Net Benefit to Employee if the 280G Payments are limited to the extent necessary to avoid being subject to the Excise Tax. Only if the amount calculated under (i) above is less than the amount under (ii) above will the 280G Payments be reduced to the minimum extent necessary to ensure that no portion of the 280G Payments is subject to the Excise Tax. For purposes of this Agreement, "Net Benefit" means the present value of the 280G Payments net of all federal, state, local, foreign income, employment and excise taxes. Any reduction made pursuant to this section will be made in a manner determined by the Company that is consistent with the requirements of Section 409A.

b. All calculations and determinations under this section will be made by an independent accounting firm or independent tax counsel appointed by the Company ("Tax Counsel") whose determinations shall be conclusive and binding on the Company and Employee for all purposes. For purposes of making the calculations and determinations required by this section, Tax Counsel may rely on reasonable, good faith assumptions and approximations concerning the application of Section 280G and Section 4999 of the Code. The Company and Employee shall furnish the Tax Counsel with such information and documents as Tax Counsel may reasonably request in order to make its determinations under this Section, and the costs of such determination shall be borne by the Company.

5. Governing Law; Jurisdiction and Venue. This Agreement, for all purposes, shall be construed in accordance with the laws of New York without regard to conflicts of law principles. Any action or proceeding by either of the parties to enforce this Agreement shall be brought only in a state or federal court located in the state of State of New York, New York County. The parties hereby irrevocably submit to the exclusive jurisdiction of such courts and waive the defense of inconvenient forum to the maintenance of any such action or proceeding in such venue.

6. Entire Agreement. Unless specifically provided herein, this Agreement contains all of the understandings and representations between Employee and the Company pertaining to the subject matter hereof and supersedes all prior and contemporaneous understandings, agreements, representations and warranties, both written and oral, with respect to such subject matter. The parties mutually agree that the Agreement can be specifically enforced in court and can be cited as evidence in legal proceedings alleging breach of the Agreement.

7. Modification and Waiver. No provision of this Agreement may be amended or modified unless such amendment or modification is agreed to in writing and signed by Employee and by a duly and expressly authorized representative of the Board. No waiver by either of the parties of any breach by the other party hereto of any condition or provision of this Agreement to be performed by the other party hereto shall be deemed a waiver of any similar or dissimilar provision or condition at the same or any prior or subsequent time, nor shall the failure of or delay by either of the parties in exercising any right, power or privilege hereunder operate as a waiver thereof to preclude any other or further exercise thereof or the exercise of any other such right, power or privilege.

8. Severability. Should any provision of this Agreement be held by a court of competent jurisdiction to be enforceable only if modified, or if any portion of this Agreement shall be held as unenforceable and thus stricken, such holding shall not affect the validity of the remainder of this Agreement, the balance of which shall continue to be binding upon the parties with any such modification to become a part hereof and treated as though originally set forth in this Agreement. The parties further agree that any such court is expressly authorized to modify any such unenforceable provision of this Agreement in lieu of severing such unenforceable provision from this Agreement in its entirety, whether by rewriting the offending provision, deleting any or all of the offending provision, adding additional language to this Agreement or by making such other modifications as it deems warranted to carry out the intent and agreement of the parties as embodied herein to the maximum extent permitted by law.

9. Counterparts. This Agreement may be executed in separate counterparts, each of which shall be deemed an original, but all of which taken together shall constitute one and the same instrument.

10. Section 409A.

a. General Compliance. This Agreement is intended to comply with Section 409A or an exemption thereunder and shall be construed and administered in accordance with Section 409A. Notwithstanding any other provision of this Agreement, payments provided under this Agreement may only be made upon an event and in a manner that complies with Section 409A or an applicable exemption. Any payments under this Agreement that may be excluded from Section 409A either as separation pay due to an involuntary separation from service or as a short-term deferral shall be excluded from Section 409A to the maximum extent possible. For purposes of Section 409A, each installment payment provided under this Agreement shall be treated as a separate payment. Any payments to be made under this Agreement upon a termination of employment shall only be made upon a "separation from service" under Section 409A. Notwithstanding the foregoing, the Company makes no representations that the payments and benefits provided under this Agreement comply with Section 409A and in no event shall the Company be liable for all or any portion of any taxes, penalties, interest or other expenses that may be incurred by Employee on account of non-compliance with Section 409A.

b. Specified Employees. Notwithstanding any other provision of this Agreement, if any payment or benefit provided to Employee in connection with [his / her] termination of employment is determined to constitute "nonqualified deferred compensation" within the meaning of Section 409A and Employee is determined to be a "specified employee" as defined in Section 409A(a)(2)(b)(i), then such payment or benefit shall not be paid until the first payroll date to occur following the six-month anniversary of the Termination Date or, if earlier, on Employee's death (the "Specified Employee Payment Date"). The aggregate of any payments that would otherwise have been paid before the Specified Employee Payment Date shall be paid to Employee in a lump sum on the Specified Employee Payment Date and thereafter, any remaining payments shall be paid without delay in accordance with their original schedule.

11. Successors and Assigns. This Agreement is personal to Employee and may not be assigned by Employee. Any purported assignment by Employee shall be null and void ab initio. The Company may assign this Agreement to any successor or assign (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business or assets of the Company. This Agreement shall inure to the benefit of the Company and its affiliates, successors and assigns.

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first above written.

[]

ROCKET PHARMACEUTICALS, INC.

By: _____

Its _____

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of November 19, 2018 ("Effective Date") by and between REGENXBIO Inc., a corporation organized under the laws of the State of Delaware, with offices at 9600 Blackwell Road, Suite 210, Rockville, MD 20850 ("Licensor"), and Rocket Pharmaceuticals, Ltd., a Cayman Islands exempted company, with offices at 350 Fifth Avenue, Suite 7530, New York, NY 10118 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to certain recombinant adeno-associated virus vectors; and

WHEREAS, Licensee desires to obtain from Licensor certain licenses under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "***" means (a) the recombinant adeno-associated virus serotype *** vector with the specified sequence set forth in GenBank (***) and (b) any recombinant adeno-associated virus derivatives of such serotype *** vector that are covered by the claims of the Licensed Patents.

1.2 "AAV9" means (a) the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank (protein id ***) and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector that are covered by the claims of the Licensed Patents.

1.3 "***" means (a) the recombinant adeno-associated virus serotype *** vector with the specified sequence set forth in GenBank (***) and (b) any recombinant adeno-associated virus derivatives of such serotype *** vector that are covered by the claims of the Licensed Patents.

1.4 "Affiliate" means any legal entity directly or indirectly, during the term of this Agreement, controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity. An entity may be or become an Affiliate of an entity and may cease to be an Affiliate of an entity, in each case, during the term of this Agreement.

1.5 "BLA" means a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2 or a New Drug Application filed with the FDA as described in 21 C.F.R. § 314.

1.6 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.7 “Change of Control” means (i) any transaction or series of related transactions following which the holders of Licensee’s capital stock or membership or equity interests immediately prior to such transaction or series of related transactions collectively are the owners of less than 50% of the outstanding equity interests of Licensee entitled to (a) vote with respect to the election of directors (or positions having a similar function) or (b) receive the proceeds upon any sale, liquidation or dissolution of Licensee; (ii) a sale, transfer, or other disposition, in a single transaction or series of related transactions, of all or a material portion of Licensee’s interest in the Licensed Products; (iii) a sale, transfer, or other disposition, in a single transaction or series of related transactions, of all or a material portion of Licensee’s right title, or interest in its assets taken as a whole; or (iv) the merger of Licensee with a Third Party by operation of law or otherwise.

1.8 “Commercial Option” has the meaning set forth in Section 2.2.

1.9 “Confidential Information” means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.7 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.9.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.9.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.9.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.9.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.9.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

- 1.10 “Disclosing Party” has the meaning set forth in Section 5.1.
- 1.11 “Domain Antibody” means * * *.
- 1.12 “EMA” means the European Medicines Agency and any successor agency(ies) in the European Union with responsibilities comparable to those of the European Medicines Agency.
- 1.13 “FDA” means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.
- 1.14 “Field” means the treatment of Danon Disease in humans by in vivo gene therapy using the Licensed Vector(s) to deliver any known LAMP2 transgene isoforms * * * and all possible combinations of LAMP2 transgene isoforms.
- 1.15 “GSK Agreement” means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as further amended from time to time.
- 1.16 “Know-How” means any and all ideas, information, know-how, data, research results, writings, inventions, discoveries, and other technology (including any proprietary materials), whether or not patentable or copyrightable.
- 1.17 “Licensed Back Improvements” means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.
- 1.18 “Licensed Know-How” means any Know-How Licensor provides to Licensee pursuant to Section 2.8.
- 1.19 “Licensed Patents” means, on a Licensed Vector-by-Licensed Vector basis, to the extent they cover such Licensed Vector, (a) all United States patents and patent applications listed in Exhibit A-1 (for AAV9), Exhibit A-2 (if the Licensed Vector is * * *), or Exhibit A-3 (if the Licensed Vector is * * *), including patents arising from such patent applications; and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that “Licensed Patents” will not include any claim of a patent or patent application covering any Manufacturing Technology.
- 1.20 “Licensed Product” means (a) any product using the applicable Licensed Vector that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, where (i) the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim of the Licensed Patents in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe or is covered by at least one Valid Claim of the Licensed Patents in the country of manufacture, use, sale, offer for sale, or import or (ii) that incorporates, was developed using, or is produced or manufactured through the use of, or with respect to which Licensee otherwise acquired a license to, Licensed Know-How; or (b) any service sold by Licensee, its Affiliates, and any of its or their Sublicensees with respect to the administration of any product using a Licensed Vector to patients that (i) in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim of the Licensed Patents in the country of sale or (ii) that incorporates, was developed using, or is produced or manufactured through the use of, or with respect to which Licensee otherwise acquired a license to, Licensed Know-How.

- 1.21 “Licensed Program” means the program relating to * * * and/or * * * for which Licensee has exercised a Commercial Option in accordance with Section 2.2.1.
- 1.22 “Licensed Technology” means, collectively, the Licensed Patents and Licensed Know-How.
- 1.23 “Licensed Vectors” means AAV9 and, if and when a Commercial Option is exercised, the vector(s) that is/are selected by Licensee pursuant to Section 2.2 (each vector, a “Licensed Vector”).
- 1.24 “Licensee Inventions” means: (i) any new or improved * * *, AAV9, and * * * capsid-related Know-How that is related specifically to the capsid of * * *, AAV9, and/or * * * and that is first discovered, produced, conceived, or reduced to practice by or on behalf of Licensee, its Affiliates, or any of its or their Sublicensees and used specifically in the development of Licensed Products in the exercise of any rights granted under this Agreement.
- 1.25 “Manufacturing Technology” means any and all patents, patent applications, Know-How, and all intellectual property rights associated therewith that are owned or controlled by Licensor, and including all tangible embodiments thereof, that claim, cover or relate to the manufacture of: adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, manufacture formulations, quality control data, specifications, scale up methods, any and all manufacture improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided by Licensor to regulatory authorities and the information contained therein are deemed Manufacturing Technology.
- 1.26 “Marketing Authorization” means all approvals, licenses, registrations or authorizations of any federal, state or local regulatory agency, department, bureau or other governmental entity, necessary for the manufacturing, use, storage, import, transport, marketing and sale of Licensed Products in a country or regulatory jurisdiction.
- 1.27 “Net Sales” means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of “Licensed Product”) by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: * * *. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm’s length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

- 1.28 “Option Term” means the period beginning on the Effective Date and ending on the fourth anniversary of the Effective Date.
- 1.29 “Penn Agreement” means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, by that certain Second Amendment to License Agreement effective on September 9, 2014, and by that certain Third Amendment to License Agreement effective on April 29, 2016, and as further amended from time to time.
- 1.30 “Phase 3 Clinical Trial” means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an BLA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.
- 1.31 “Prosecute” means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences.
- 1.32 “Receiving Party” has the meaning set forth in Section 5.1.
- 1.33 “REGENXBIO Licensors” means SmithKline Beecham Corporation (or any successor thereto under the GSK Agreement) and The Trustees of the University of Pennsylvania (or any successor thereto under the Penn Agreement), if any Licensed Technology is sublicensed from the Penn Agreement.
- 1.34 “Retained Rights” has the meaning set forth in Section 2.3.
- 1.35 “Sublicensee” means (i) any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement; and (ii) any other Third Party or Affiliate to whom a sublicensee described in clause (i) has granted a further sublicense as permitted by this Agreement.
- 1.36 “Third Party” means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.
- 1.37 “Valid Claim” means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, that has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction and has not been pending for more than fifteen (15) years.

ARTICLE 2: LICENSE GRANTS

2.1 License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.5 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products using AAV9 solely in the Field, including, for the avoidance of doubt, the right to conduct research and development.

2.2 Commercial License Option. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee during the Option Term two distinct exclusive options, one of which is using * * * in the Field and one of which is using * * * in the Field, exercisable at Licensee's sole discretion, to obtain an exclusive, sublicensable (as provided in Section 2.5 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products (each such right, a "Commercial Option") in accordance with the following provisions:

2.2.1 Method of Exercise. To exercise a Commercial Option, Licensee must, prior to the end of the Option Term, (i) provide written notice to Licensor, which written notice must specify which Commercial Option is being exercised and (ii) pay the fee specified in Section 3.2.

2.2.2 License Grant Upon Exercise. If Licensee exercises a Commercial Option, effective upon Licensor's receipt of the notice and, the fee described in Section 2.2.1 (the "Grant Date" for the applicable Licensed Program), subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor shall be deemed to have granted to Licensee an exclusive, sublicensable (as provided in Section 2.5 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products in the Field that are the subject of the Licensed Program, including, for the avoidance of doubt, the right to conduct research and development.

2.2.3 Exercised Commercial Option. For the avoidance of doubt, the foregoing license granted pursuant to Section 2.2.2 will be deemed granted on the Grant Date on a Licensed Program-by-Licensed Program basis, solely with respect to the Licensed Program for which a particular Commercial Option was exercised under this Section 2.2. The Parties acknowledge that there may be different Grant Dates for each Licensed Program, depending on when and if Licensee exercises a Commercial Option for the applicable Licensed Program. Each Commercial Option will terminate at the end of the Option Term if Licensee fails to exercise such Program Option prior to the end of the Option Term, and Licensee will have no further rights under this Agreement to exercise any unexercised Program Option.

2.3 **Retained Rights.** Except for the rights and licenses specified in Section 2.1 and, if applicable, Section 2.2.2, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise and whether such intellectual property is subordinate, dominant, or otherwise useful for the practice of the Licensed Technology. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Technology for any research, development, commercial, or other purposes outside of the Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees that the following rights are retained by Licensor and the REGENXBIO Licensors (individually and collectively, the “Retained Rights”), whether inside or outside the Field:

2.3.1 The rights and licenses granted in Section 2.1 and, if applicable, Section 2.2.2, shall not include any right (and Licensor and the REGENXBIO Licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including any Licensed Vector.

2.3.2 Licensor and the REGENXBIO Licensors retain the following rights with respect to the Licensed Technology:

- (a) A non-exclusive, sublicensable right under the Licensed Technology to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including any Licensed Vector; and
- (b) A non-exclusive right for the REGENXBIO Licensors (which right is sublicensable by the REGENXBIO Licensors) to use the Licensed Technology for non-commercial research purposes and to use the Licensed Technology for such REGENXBIO Licensors’ discovery research efforts with non-profit organizations and collaborators.

2.3.3 The rights and licenses granted in Section 2.1 and, if applicable, Section 2.2.2, shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology:

- (a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field; or
- (b) to use the Licensed Technology to provide services to any Third Parties; provided that Licensee’s license under Section 2.1 and, if applicable, Section 2.2.2, does include the right to provide the service of the administration of Licensed Products to patients.

2.3.4 Licensor retains the fully sublicensable right under the Licensed Technology to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such research and development rights retained by Licensor shall not include the right to conduct (or permit others to conduct) clinical trials in humans in the Field or any rights to use the Licensed Technology to sell products in the Field.

2.3.5 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Technology for educational and non-commercial research purposes.

2.4 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grants hereunder are expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.5 Sublicensing.

2.5.1 The license granted pursuant to Section 2.1 and, if applicable, Section 2.2.2, is sublicensable by Licensee to any Affiliates or Third Parties (through multiple tiers); provided that any such sublicense must comply with the provisions of this Section 2.5 (including Section 2.5.2).

2.5.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

- (a) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee. Licensor must receive written notice as soon as practicable following execution of any such sublicenses. Any further sublicenses granted by any Sublicensees (to the extent permitted hereunder) must comply with the provisions of this Section 2.5 (including Section 2.5.2) to the same extent as if Licensee granted such sublicense directly.
- (b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.
- (c) The official language of any sublicense agreement shall be English.
- (d) Within * * * after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with the REGENXBIO Licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or the REGENXBIO Licensors') ability to ensure compliance with this Agreement; provided that, if either of the REGENXBIO Licensors requires a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

- (e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain *** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.6 Non-Exclusive License Under Licensee Inventions. Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license under Licensee Inventions (and any intellectual property rights with respect thereto) to practice the Licensee Inventions solely in connection with AAV9, (or *** or *** if Licensee exercises the Commercial Option) and solely outside of the Field, including, for avoidance of doubt, the right to conduct research and develop and commercialize products and services outside of the Field.

2.7 Improvements.

2.7.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

- (a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and
- (b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with ***, AAV9 and ***, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that Licensor shall have no right, under the license in this Section 2.7.1(b), to practice the Licensed Back Improvements in the Field.

2.7.2 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensee Invention and/or any Licensed Back Improvement, together with a reasonably detailed description of, or access to, such Licensee Inventions and/or Licensed Back Improvement to permit the practice of any such invention or improvement.

2.8 Transfer of Licensed Know-How. Following the Effective Date and from time to time during the Term, at Licensee's sole expense and request, Licensor may agree at its sole discretion to disclose to Licensee within *** following such written request from Licensee manufacturing and development Know-How, including through in-person or telephonic meetings at such times and places as agreed to by the Parties, necessary or reasonably useful to make or practice the Licensed Vectors that Licensor agrees to provide to Licensee. Licensee acknowledges and agrees that all Licensed Know-How disclosed pursuant to this Section 2.8 will be deemed "Confidential Information" of Licensor, regardless of whether such information is marked or identified as confidential and without an obligation to summarize oral information.

2.9 Section 365(n) of the Bankruptcy Code. All rights and licenses granted to Licensee or Licensor under or pursuant to this Agreement are and will otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended (the "Bankruptcy Code") or any comparable law outside the United States, licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code and any comparable law outside the United States.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor an initial fee of \$7,000,000 within * * * of the Effective Date.

3.2 Commercial Option Fee. Licensee shall pay Licensor a fee of \$2,000,000 within * * * of the exercise of each Commercial Option.

3.3 Annual Maintenance Fee. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor on-going annual maintenance fees of * * * per Licensed Vector on each anniversary of the Effective Date. For clarity, the maintenance fee for * * * will not be due unless the respective Commercial Options have been exercised.

3.4 Milestone Fees. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor the following milestone payments within * * * of each Licensed Product containing a distinct Licensed Vector (specifically, * * *, AAV9 * * *), to achieve such milestone event:

<u>Milestone</u>	<u>Milestone Payment</u>
1. First treatment of human subject in the first clinical trial (<i>i.e.</i> , first patient, first dose)	* * *
2. First treatment of first human subject in Phase 3 Clinical Trial (<i>i.e.</i> , first patient, first dose)	* * *
3. BLA application submitted to the FDA in the United States	* * *
4. Marketing Authorization application submitted to the EMA in the European Union	* * *
5. BLA approval received from the FDA in the United States	* * *
6. Marketing Authorization approval received from the EMA in the European Union	* * *
Total (per Licensed Product):	\$13,000,000

3.4.1 For clarity, the milestone payments set forth in Section 3.4 are payable regardless of whether the milestone is achieved by Licensee, any Affiliate, or any Sublicensee, and regardless of whether the milestone is achieved by more than one Licensed Product with distinct Licensed Vectors. To the extent that either of the first two development milestones in Section 3.4 (i.e., first treatment of a human subject in a clinical trial or first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of either the BLA or Marketing Authorization submission milestone, then, upon the achievement of either of such submission milestones, the preceding unpaid development milestone payments shall be made in addition to the payment corresponding to the applicable submission milestone that has been achieved.

3.5 Royalties. In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay to Licensor the following royalties based upon the annual Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.5.1:

<u>Cumulative Annual Net Sales of all Licensed Products Worldwide</u>	<u>Royalty Percentage</u>
Portion of Annual Net Sales less than \$***	***
Portion of Annual Net Sales greater than (and including) \$***	***

For clarity, the Annual Net Sales resets to zero at the beginning of each calendar year.

3.5.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from one or more Third Parties to avoid infringement of such Third Party(ies)'s rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party(ies) for such license, together with those royalties payable to Licensor, in the aggregate, exceed *** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

$$R = (C * (A / (A+B)))$$

Where

R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ***.

Example Calculation:

- Assume:
- i) all Third Party royalties = * * *
 - ii) unreduced Licensor royalty = * * *
 - iii) projected total royalty = * * *

R = * * *

R = * * *

R = * * *

Licensor Stacked Royalty = * * * (but subject to the cap described below)

Notwithstanding the foregoing, Licensee will pay to Licensor no less than * * * of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.5.2 Adjustment of Royalties For Licenses. On a Licensed Product-by-Licensed Product, country-by-country basis, upon the date on which the manufacture, use, sale, offer for sale, or import of a Licensed Product does not infringe or is not covered by a Valid Claim in such country, then the royalty percentage applicable to Net Sales of such Licensed Product under this Section 3.5 in such country shall be reduced by * * *.

3.5.3 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.5 on the Net Sales of Licensed Products in a given country will end on a country-by-country, Licensed Product-by-Licensed Product basis on the later of: (i) expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Patents to expire, lapse, become abandoned or become unenforceable for the applicable Licensed Product in the applicable country, or (ii) * * * from the first commercial sale of the applicable Licensed Product in the applicable country.

3.6 Sublicense Fees & Priority Review Voucher Transfer Fee.

3.6.1 In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee will pay Licensor * * * of any sublicense fees (* * *) received by Licensee or its Affiliates from a Third Party for the Licensed Technology from any Sublicensee or from any person or entity granted any option to obtain a sublicense ("Sublicensing Revenue").

3.6.2 In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee will pay Licensor twenty percent (20%) of the fees related to the sale or other transfer or rights to a Third Party under any priority review voucher issued in connection with or otherwise related to a Licensed Product. Should Licensee or its Affiliates elect to use any priority review voucher issued in connection with or otherwise related to a Licensed Product rather than sell or transfer the priority review voucher to a Third Party, Licensee will pay Licensor 20% of the average value of the last five publicly disclosed priority review voucher sales at the time of use.

3.6.3 With respect to the obligations under this Section 3.6, Licensee shall not be required to submit any amounts received from a Third Party for the following:

- (a) Reimbursement or payment of Licensee's actual costs or on an arm's length cost plus arrangement for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the research, development and/or manufacturing of Licensed Products pursuant to a specific agreement;
- (b) Any and all amounts paid to Licensee or its Affiliates by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement; and
- (c) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value or in the form of loans at arm's length rates of interest.

3.6.4 If Licensee or its Affiliates receives sublicense fees from Sublicensees or from any person or entity granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then Licensee shall pay Licensor a cash payment as required under Section 3.6 determined based on the fair market value of such non-cash consideration. If Licensee or its Affiliate enters into any sublicense that is not an arm's length transaction, fees due under this Section 3.6 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

3.6.5 To the extent Licensee or its Affiliates receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.4, then the amount of the payment made to Licensor under such Section 3.4 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.6; instead, the amounts due under this Section 3.6 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.6 above to the sublicense fees received by Licensee or its Affiliates from such Third Party after deducting the amount of the payment under Section 3.4.

3.7 Reports and Records.

3.7.1 Licensee must deliver to Licensor within * * * after the end of each Calendar Quarter beginning after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

- (a) Number of Licensed Products included within Net Sales, listed by country;
- (b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received, listed by country;
- (c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.25, listed by category of cost and by country;

- (d) Net Sales of Licensed Products listed by country;
- (e) A detailed accounting of any royalty reductions applied pursuant to Section 3.5.1;
- (f) Royalties owed to Licensor; and
- (g) The computations for any applicable currency conversions.

3.7.2 Licensee shall pay the royalties due under Section 3.5 within * * * following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.7.1.

3.7.3 Within * * * after the occurrence of a milestone event described in Section 3.4, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.4.

3.7.4 Within * * * after the receipt of any fees from any Third Party as described in Section 3.6, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.6.

3.7.5 All financial reports under this Section 3.7 will be certified by the chief financial officer of Licensee or Licensee's qualified financial representative.

3.7.6 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete, and accurate books and records that enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for * * * after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or the REGENXBIO Licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of five years thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by * * * or more, then Licensee will also promptly pay the costs and expenses of Licensor and the REGENXBIO Licensors and their respective accountants in connection with the review or audit. If the review or audit determines that Licensee has overpaid any payment, then Licensor shall refund the overpayment to Licensee.

3.8 Currency, Interest.

3.8.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.8.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal*, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.7.

3.8.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.9 Taxes and Withholding.

3.9.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.9.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.9.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, then Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ***. Without limiting the foregoing, Licensee will meet the following: acceptance by the FDA or foreign equivalent of an investigational new drug application, for a Licensed Product by no later than *** after the Effective Date; provided, however, that, if Licensee expects not to achieve the milestone on or before the specified deadline, Licensee may pay Licensor an extension fee of *** on or before such deadline and the deadline shall then be extended by an ***. Licensee will provide Licensor written notice within *** of achieving the milestone.

4.2 Development Plans.

4.2.1 For each Licensed Product in the Field, Licensee will prepare and deliver to Licensor a development plan and budget (each a "Development Plan"). The initial Development Plans for the Licensed Product in the Field will be delivered within * * * after the Effective Date.

4.2.2 Each Development Plan will cover the next 2 years, and will include future development activities that Licensee plans to undertake (either directly or through its Affiliates, or any Sublicensees) during those 2 years that relate directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy.

4.2.3 Following receipt by Licensor of each Development Plan, Licensor will promptly notify Licensee of any comments or suggested revisions, and Licensee shall incorporate any appropriate comments and suggested revisions in good faith.

4.3 Reporting. Within * * * after the Effective Date and within * * * thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, a summary of the progress of the development, evaluation, testing and commercialization of each Licensed Product. Licensee will also notify Licensor within * * * of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.3.1 Date of Development Progress Report and time covered by such report;

4.3.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Products since the last Development Progress Report;

4.3.3 Significant research and development projects relating directly to the Licensed Products currently being performed by Licensee, its Affiliates, and any Sublicensees and good faith, but non-binding, projected dates of completion;

4.3.4 A summary update to the Development Plan, which will include a summary of anticipated future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Products, Licensee's strategy to bring the Licensed Products to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.3.5 Projected total development remaining before product launch of each Licensed Product; and

4.3.6 Summary of significant development efforts using the Licensed Technology being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.4 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with the REGENXBIO Licensors.

4.5 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensee Inventions and any Licensed Back Improvements, if not previously provided pursuant to Section 2.7.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) protect the Confidential Information of the Disclosing Party with at least the same degree of care as it protects its own confidential and proprietary information, and in any event with not less than a reasonable degree of care; (c) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder shall be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (d) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties may agree to issue a joint press release. Except as provided in Section 5.2.2, any press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once a joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or Licensed Vector program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose the other's Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any * * *; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder, with the REGENXBIO Licensors to the extent required by the GSK Agreement and the Penn Agreement, under confidentiality. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law, rule, regulation or rules of a security exchange, to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, then, to the extent legally permitted, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will, at the Disclosing Party's request and expense, provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law, rule, regulation or rules of a security exchange, to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, then the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of * * * following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement will commence on the Effective Date and continue in effect on a country-by-country, Licensed Product-by-Licensed Product basis until the later of: (i) the expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Patents to expire, lapse, become abandoned, become unenforceable for the applicable Licensed Product, or (ii) 10 years from the first commercial sale of a Licensed Product, unless sooner terminated as provided in this Agreement. Upon expiration of the Agreement, the license grant to Licensee pursuant to Section 2.1 shall become irrevocable, perpetual, royalty-free and fully paid-up.

6.2 Termination for Failure to Exercise Commercial Option(s). Each Commercial Option will terminate automatically if such Commercial Option has not been exercised at the end of the Option Term; provided that such termination will not affect Licensee's rights under this Agreement with respect to the license granted under Section 2.1.

6.3 Licensee's Right to Terminate. Licensee may, upon * * * prior written notice to Licensor, terminate this Agreement for any reason, with or without cause. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to one or more Licensed Vectors in the Field.

6.4 Termination for Breach.

6.4.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and if Licensee does not pay Licensor in full within * * * upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such * * * cure period.

6.4.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within * * * after written notice of the breach, which termination shall be effective immediately upon the expiration of such * * * cure period. Notwithstanding the above, if Licensee disputes in good faith that such material breach exists, and gives Licensor written notice of such dispute within * * * following Licensee's receipt of Licensor's notice of default, then, Licensor may not terminate this agreement until the dispute is resolved in accordance with Section 10.6; provided that Licensor shall be entitled to terminate this Agreement at the end of the original * * * cure period, without waiting for resolution of the dispute in accordance with Section 10.6, if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6.5 Termination for Insolvency. Licensor shall have the right to terminate this Agreement, upon notice to the Licensee, in the event that:

(a) Licensee shall have: (i) voluntarily commenced any proceeding or filed any petition seeking relief under the bankruptcy, insolvency or other similar laws of any jurisdiction, (ii) applied for, or consented to, the appointment of a receiver, trustee, custodian, sequestrator, conciliator, administrator or similar official for it or for all or substantially all of its property, (iii) filed an answer admitting the material allegations of a petition filed against or in respect of it in any such proceeding, (iv) made a general assignment for the benefit of creditors of all or substantially all of its assets, (v) admitted in writing its inability to pay all or substantially all of its debts as they become due, or (vi) taken corporate action for the purpose of effecting any of the foregoing; or

(b) An involuntary proceeding shall have been commenced, or any involuntary petition shall have been filed, in a court of competent jurisdiction seeking: (i) relief in respect of Licensee, or of its property, under the bankruptcy, insolvency or similar laws of any jurisdiction, (ii) the appointment of a receiver, trustee, custodian, sequestrator, conciliator, administrator or similar official for the Licensee or for all or substantially all of its property, or (iii) the winding-up or liquidation of the Licensee; and, in each case, such proceeding or petition shall have continued undismissed for 60 days, or an order or decree approving or ordering any of the foregoing shall have continued unstayed, unappealed and in effect for 30 days.

6.6 Patent Challenge.

6.6.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.6.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. If a Sublicensee commences a Patent Challenge and Licensee fails to terminate the applicable sublicense agreement, then Licensor may terminate this Agreement, effective immediately upon written notice to the Licensee.

6.6.3 For purposes of this Section 6.6, “Patent Challenge” means any action against Licensor or the REGENXBIO Licensees, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.7 Effects of Termination. The effects of termination by Licensee pursuant to Section 6.3, by either Party, as applicable, pursuant to Section 6.4, or by Licensor pursuant to Section 6.5 or 6.6 shall be as follows:

6.7.1 The applicable licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.7.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Technology; provided that Licensee, its Affiliates, and its Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products under the terminated licenses for a period not to exceed * * * after the effective date of such termination;

6.7.2 If termination is by Licensor pursuant to Sections 6.4, 6.5 or 6.6, then, at Licensor’s request, Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor’s obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate. If termination is for any other reason, then all sublicenses shall terminate;

6.7.3 If termination is by Licensee pursuant to Section 6.3 or by Licensor pursuant to Section 6.4, 6.5, or 6.6, then Licensee shall grant, and hereby grants, to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any Licensed Back Improvements, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.7.4 Licensee shall pay all monies then-owed to Licensor under this Agreement;

6.7.5 Each Receiving Party shall, at the Disclosing Party’s request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party’s obligations; and

6.7.6 If termination is only with respect to one or more Licensed Vectors within the Field, but not all Licensed Vectors, then the provisions of this Section 6.7 shall only apply with respect to the terminated Licensed Vectors, and this Agreement shall continue with respect to the non-terminated Licensed Vectors.

6.8 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement that have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.3, (Retained Rights), Section 2.4 (Government Rights), Section 2.6 (Non-Exclusive License Under Licensee Inventions), Section 2.7 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.7 (Reports and Records), Section 4.4 (Confidential Information), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions, inter partes review, or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that The Trustees of the University of Pennsylvania control Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of the REGENXBIO Licensors set forth in the GSK Agreement and Penn Agreement with respect to the Licensed Patents, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under the GSK Agreement and the Penn Agreement. Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention, including any "patent certification" filed in the United States under 21 U.S.C. § 355(b)(2) or 21 U.S.C. § 355(j)(2) or similar provisions in other jurisdictions alleging the invalidity, unenforceability or non-infringement of any Licensed Patents, and any notification received pursuant to subsection (k) of 42 U.S.C § 262 for any Licensed Product that becomes a "reference product." However, Licensee is under no obligation to search for potential infringers.

7.2.2 As between Licensor and Licensee, but subject to any obligations of Licensor to the REGENXBIO Licensors, Licensor shall have the sole right, but not the obligation, to prosecute any such infringement * * *. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action. Nothing in this Agreement obligates Licensor to bring or prosecute lawsuits against Third Parties for infringement of any Licensed Patents.

7.2.3 Licensee shall have no right to undertake prosecution of any such infringement.

7.3 **Defense of Infringement Claims.** In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another. To the extent Licensor takes any action, Licensor (or the REGENXBIO Licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, not to be unreasonably withheld, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or the REGENXBIO Licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 **Representations and Warranties by Licensor.** Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.1.4 To Licensor's knowledge, (a) the Licensed Patents are solely owned by The Trustees of the University of Pennsylvania, and (b) no Third Party (other than the REGENXBIO Licensors) has any right, interest, or claim in or to such Licensed Patents in the Field that are inconsistent with those granted to Licensee in the Field under this Agreement;

8.1.5 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Patents in the Field; and

8.1.6 To Licensor's knowledge, Licensor does not Control as of the Effective Date any patent or patent application (other than the Licensed Patents (as defined in Section 1.17) that would necessarily be infringed by the use or sale of Licensed Vectors in the Field. If it is determined, in accordance with the procedure of this Section 8.1.6, that Licensor Controls as of the Effective Date a patent or patent application (other than the Licensed Patents) that would necessarily be infringed by the use or sale of Licensed Vectors in the Field, then Licensee's sole remedy shall be the inclusion of the applicable patent or patent application as a "Licensed Patent" hereunder but solely to the extent of the claim(s) that would necessarily be infringed by the use or sale of Licensed Vectors. At any time during the term of this Agreement, Licensee may notify Licensor in writing of any such patent or patent application that Licensee believes should be included as a "Licensed Patent" pursuant to this Section 8.1.6. Such written notice shall identify the relevant patent or patent application and relevant claim(s) and shall explain briefly why Licensee, in good faith, believes it should be included as a "Licensed Patent." Licensor has * * * following Licensor's receipt of Licensee's written notice to dispute the inclusion of such patent or patent application or the scope of the remedy; in which event, such dispute will be resolved in accordance with Section 10.6. Upon the Parties' agreement (or a resolution, in favor of Licensee, of the dispute pursuant to Section 10.6), the applicable claim(s) of the applicable patent or patent application will be deemed a "Licensed Patent" hereunder. For the avoidance of doubt, Licensor makes no representation or warranty under this Section 8.1.6 as to any claim of a patent or patent application covering the manufacture of Licensed Patents, and Licensee acknowledges that manufacturing claims of any patents or patent applications will not be added as "Licensed Patents" pursuant to the procedure set forth in this Section 8.1.6. For the purpose of this Section 8.1.6, "Control" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent or patent application on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any Third Party.

8.2 Representations and Warranties by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact Licensee's activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, EXCEPT AS SET FORTH IN SECTION 8.1, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, TITLE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED TECHNOLOGY, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED TECHNOLOGY, OR LICENSED PRODUCTS WILL NOT INFRINGE ANY TECHNOLOGY, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR AND THE REGENXBIO LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, EXEMPLARY, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, the REGENXBIO Licensors, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: * * *; provided, however, that Licensee shall not be liable for claims to the extent based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

- (a) any *** or other claim of any kind related to the *** by a Third Party of a Licensed Product that *** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;
- (b) any claim by a Third Party that the ***; and
- (c) *** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Technology or Licensed Products, including any claim by or on behalf of a ***.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, contractors, agents, and employees (individually, a "Licensee Indemnified Party," and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that result from or arise out of: (i) any breach by Licensor of the representations, warranties, or obligations of this Agreement; and (ii) Licensor's or its Affiliates' gross negligence or intentional misconduct; provided, however, that Licensor shall not be liable for claims based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on any indemnified party (an "Indemnified Party") without the other Party's prior written consent or, if Licensee is the Indemnifying Party, that grants any rights to the Licensed Technology or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. Upon the Indemnifying Party's reasonable request, the Indemnified Parties will reasonably cooperate with the Indemnifying Party in the defense and settlement of any such claim, at the Indemnifying Party's cost and expense. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within *** after notice thereof, then the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of an Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within *** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 **Insurance.** Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of * * * combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than * * * (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of * * * combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until * * * after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of * * * combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of * * * or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within * * * after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least * * * prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

9.1 Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable, unless required to do so pursuant to applicable law, rule, regulation or rules of a securities exchange; provided, however that Licensee may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable.

9.2 Licensor and all of its employees and agents must not use Licensee's name, seal, logo, trademark, or service mark (or any adaptation thereof) in any way without the prior written consent of Licensee; provided, however that Licensor may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable, and refer to Licensee as a licensee of Licensor.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign or otherwise transfer (by operation of law or otherwise) this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor, which consent is in the absolute discretion of Licensor but shall not be unreasonably withheld (except Licensee shall have the right to assign this Agreement without Licensor's consent to a wholly owned Affiliate, in which case Licensee shall remain responsible for the performance of this Agreement by such Affiliate); provided, however, Licensee shall be permitted to transfer (by operation of law or otherwise) this Agreement without Licensor's consent in connection with a Change of Control; provided that, Licensee: (i) requires any transferee or successor to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such undertaking; (ii) provides Licensor with written notice of the Change of Control to Licensor within * * * of the consummation of the transaction resulting in a Change of Control of Licensee; and (iii) provides Licensor with a copy of the definitive agreement for the Change of Control of Licensee within * * * of the consummation of the transaction (provided, that Licensee shall be entitled to include customary redactions in such copy provided to Licensor, to the extent such redacted information is not necessary to verify compliance with the terms of this Agreement or otherwise required by the Penn Agreement and/or GSK Agreement). Notwithstanding anything to the contrary in this Agreement, for clarity, in case of a Licensee Change of Control, in no event shall any intellectual property rights owned or controlled by the acquirer or its Affiliates immediately prior to such Licensee Change of Control be included in any of the licenses granted to Licensor under this Agreement. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations that it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, by facsimile, or by electronic mail (with a copy of such facsimile or electronic mail also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

REGENXBIO Inc.
9600 Blackwell Road
Suite 210
Rockville, MD 20850
USA
Attn: Chief Executive Officer
Telephone: 240-552-8181
Facsimile: 240-652-9692

with a copy to:

REGENXBIO Inc.
9600 Blackwell Road
Suite 210
Rockville, MD 20850
USA
Attn: General Counsel
Telephone: 240-552-8181
Facsimile: 240-652-9692

If for Licensee:

Rocket Pharmaceuticals, Inc.
350 Fifth Avenue
Suite 7530
New York, NY 10118
USA
Attn: Chief Executive Officer
Telephone: 646-440-9100
Facsimile:

with a copy to:

Rocket Pharmaceuticals, Inc.
350 Fifth Avenue
Suite 7530
New York, NY 10118
USA
Attn: Director of Legal & Corporate Development
Telephone: 646-440-9100
Facsimile:

Either Party may change its official address upon written notice to the other Party in accordance with this Section 10.4.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than * * * following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within * * * after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within * * * from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and Licensee shall use reasonable efforts to require that any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement dated * * * between the Parties. All "Confidential Information" (as defined in such Mutual Non-Disclosure Agreement) disclosed by one Party to the other Party pursuant to such Mutual Non-Disclosure Agreement shall be deemed "Confidential Information" of such disclosing Party under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.9). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, then the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other reasonable acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word “including” shall be deemed to be followed by the phrase “without limitation” or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words “herein” or “hereunder” relate to this Agreement; (e) “or” is disjunctive but not necessarily exclusive; (f) the word “will” shall be construed to have the same meaning and effect as the word “shall”; (g) all references to “dollars” or “\$” herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections, Articles, and exhibits in this Agreement are to Sections, Articles, and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENXBIO INC.

ROCKET PHARMACEUTICALS, LTD.

By: /s/ Kenneth Mills

Name: Kenneth Mills

Title: President and CEO

By: /s/ Gaurav D. Shah

Name: Gaurav D. Shah

Title: CEO and President



Rocket Pharmaceuticals and REGENXBIO Announce New License Agreement for the Treatment of Danon Disease Using NAV AAV9 Vector

- REGENXBIO grants Rocket exclusive, worldwide rights to NAV AAV9 for the development and commercialization of treatments for Danon Disease -

- Investigational New Drug Application Expected to be Filed in 2019; Clinical Trial to Follow -

NEW YORK and ROCKVILLE, Md. – November 26, 2018 - Rocket Pharmaceuticals, Inc. (Nasdaq: RCKT), a leading U.S.-based multi-platform gene therapy company, and REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV[®] Technology Platform, today announced an exclusive, worldwide license agreement for Rocket to develop and commercialize gene therapy treatments for Danon disease using REGENXBIO's NAV AAV9 vector, as well as exclusive options for two additional NAV AAV vectors for the treatment of Danon disease.

Under the terms of the agreement, REGENXBIO has granted Rocket an exclusive, worldwide license, with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapy treatments for Danon disease. REGENXBIO has also granted Rocket exclusive options to two additional undisclosed NAV AAV vectors for the treatment of Danon disease for up to four years. In return for these rights, REGENXBIO will receive a \$7 million upfront payment, ongoing fees, milestone payments and high-single to low-double digit royalties on net sales of products incorporating the licensed intellectual property.

"Rocket is delighted to partner with REGENXBIO to advance our first gene therapy product derived from the NAV Technology Platform, reinforcing the potential of our disease-focused, multi-platform development approach allowing for the potential first-mover advantage without being limited by vector type," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Danon disease represents a significant area of unmet medical need as no specific therapies are available for the patients and families suffering from the disease. We believe the well-understood disease biology and severe patient need makes Danon disease an ideal target for gene therapy and we expect to enter the clinic next year."

"This license agreement provides further validation of the potential for the diverse application of our NAV Technology Platform for the treatment of severe diseases with high unmet medical," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to initiate our partnership with Rocket, an emerging leader in gene therapy for rare diseases, via development of a gene therapy treatment for Danon disease."

About Danon Disease

Danon disease is a rare neuromuscular and cardiovascular disease characterized by profound cardiomyopathy, skeletal myopathies, and mild cognitive impairment. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the European Union. Danon disease is caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. The LAMP-2 protein has three distinct variants generated by alternative splicing: LAMP-2A, LAMP 2B and LAMP-2C. Mutations resulting in LAMP-2B dysfunction are associated with severe cardiac disease features. The disease is often fatal in patients in the second or third decade of life due to progressive heart failure unless treated with a cardiac transplantation, which is nonetheless not considered curative. There are no specific therapies available for the treatment of Danon disease.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket's multi-platform development approach applies the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Preclinical studies of additional bone marrow-derived disorders are ongoing and target Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket's first AAV-based gene therapy program targets Danon disease, a rare neuromuscular and cardiovascular disease. For more information about Rocket, please visit www.rocketpharma.com.

About REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

Rocket Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding the safety, effectiveness and timing of product candidates that Rocket may develop, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe", "expect", "anticipate", "intend", "plan", "will give", "estimate", "seek", "will", "may", "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, Rocket's ability to commence a registrational study in FA within the projected time periods, the potential advantages of Rocket's product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2017. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

REGENXBIO Forward-Looking Statements

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations and clinical trials. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2017 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Rocket Contact:

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SVP, Corporate Strategy and IRO
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cp@rocketpharma.com
www.rocketpharma.com
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REGENXBIO Contacts:

Investors

Natalie Wildenradt, 646-681-8192
natalie@argotpartners.com

Media

Adam Pawluk, 202-591-4063
apawluk@jpa.com

Subsidiaries of Rocket Pharmaceuticals, Inc.

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

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. 333-225896) and Forms S-8 (Nos. 333-204501, 333-212308, 333-216892 and 333-223488) of our report dated March 8, 2019, on our audits of the consolidated financial statements as of December 31, 2018 and 2017 and for each of the years in the three-year period ended December 31, 2018, which report is included in this Annual Report on Form 10-K to be filed on or about March 8, 2019.

/s/ EISNERAMPER LLP

EISNERAMPER LLP
New York, New York
March 8, 2019

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 8, 2019

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

Certifications under Section 302

I, John Militello, 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 8, 2019

/s/ John Militello

John Militello

Controller

(Principal Financial and Accounting Officer)

**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, 2018, 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 8, 2019

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

Date: March 8, 2019

/s/ John Militello
John Militello
Controller
(Principal Financial and Accounting 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.
