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Filed Pursuant to Rule 424(b)(5)
Registration No. 333-210585

The information in this preliminary prospectus supplement is not complete and may be changed. This preliminary prospectus supplement and the accompanying prospectus do not constitute an offer to sell these securities and we are not soliciting offers to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to Completion
Preliminary Prospectus Supplement dated January 23, 2018

PROSPECTUS SUPPLEMENT
(To the Prospectus dated April 14, 2016)

Shares

Rocket Pharmaceuticals, Inc.

Common Stock

We are offering shares of our common stock, par value $0.01 per share, at a price of $ per share. Our common stock is listed on The Nasdaq Global Market under the symbol "RCKT." On January 22, 2018, the last reported sale price per share of our common stock was $13.27 per share.

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups Act of 2012, and, as such, we have elected to take advantage of reduced reporting requirements for this prospectus supplement and may elect to comply with certain reduced public company reporting requirements for future filings.

Investing in our securities involves certain risks. See "Risk Factors" beginning on Page S-11 of this prospectus supplement and page 3 of the accompanying prospectus, and in the other documents that are incorporated by reference herein and any related free writing prospectus, for certain risks you should consider. You should read the entire prospectus supplement and the accompanying prospectus, including any information incorporated by reference herein, carefully before you make your investment decision.

	Per Share	Total
Public offering price	$	$
Underwriting discounts and commissions (1)	$	$
Proceeds, before expenses, to us	$	$

(1) We have agreed to reimburse the underwriters for certain expenses. See "Underwriting."

The underwriters may also exercise their option to purchase up to an additional shares of common stock from us, at a price of $ per share, for 30 days after the date of this prospectus supplement.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus supplement or the accompanying prospectus. Any representation to the contrary is a criminal offense.

The shares will be ready for delivery on or about January , 2018.

Joint Bookrunning Managers

Cowen **Evercore ISI**

Prospectus Supplement dated January , 2018.

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You should rely only on the information contained in this prospectus supplement and the accompanying prospectus, including any information incorporated by reference, and in any free writing prospectus that we have authorized for use in connection with this offering. We have not, and the underwriters have not, authorized anyone to provide you with different information. If anyone provides you with different or inconsistent information, you should not rely on it. You should not assume that the information contained in this prospectus supplement and the accompanying prospectus, or in any free writing prospectus that we have authorized for use in connection with this offering, is accurate as of any date other than the date of those respective documents, or that information contained in any document incorporated by reference is accurate as of any date other than the date of the document incorporated by reference. Our business, financial condition, results of operations and prospects may have changed since those dates. We are not, and the underwriters are not, making offers to sell these securities in any jurisdiction in which an offer or solicitation is not authorized or permitted or in which the person making such offer or solicitation is not qualified to do so or to any person to whom it is unlawful to make such an offer or solicitation. You should read this prospectus supplement, the accompanying prospectus, including any information incorporated by reference, and any free writing prospectus that we have authorized for use in connection with this offering, in their entirety before making an investment decision. You should also read and consider the information in the documents to which we have referred you in the sections entitled “Where You Can Find Additional Information” and “Information Incorporated by Reference.”

ABOUT THIS PROSPECTUS SUPPLEMENT

This document is part of the registration statement that we filed with the Securities and Exchange Commission, or the SEC, using a "shelf" registration process, and consists of two parts. The first part is this prospectus supplement, including the documents incorporated by reference, which describes the specific terms of this offering. The second part, the accompanying prospectus, including the documents incorporated by reference, gives more general information, some of which may not apply to this offering. Generally, when we refer to the "prospectus," we are referring to both parts combined. This prospectus supplement and any free writing prospectus we authorize for use in connection with this offering may add to, update or change information in the accompanying prospectus and the documents incorporated by reference into this prospectus supplement or the accompanying prospectus.

If information in this prospectus supplement is inconsistent with the accompanying prospectus or with any document incorporated by reference herein or therein that was filed with the SEC before the date of this prospectus supplement, you should rely on the information contained in this prospectus supplement; provided that if any statement in one of these documents is inconsistent with a statement in another document having a later date, the statement in the document having the later date modifies or supersedes the earlier statement. This prospectus supplement, the accompanying prospectus, the documents incorporated by reference into each and any free writing prospectus we authorize for use in connection with this offering include important information about us, the shares and other information you should consider before purchasing the shares. See "Where You Can Find Additional Information" and "Information Incorporated by Reference" in this prospectus supplement.

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to any document that is incorporated by reference herein were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreements, and should not be deemed to be a representation, warranty or covenant to you. Moreover, such representations, warranties and covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

We have not and the underwriters have not authorized anyone to provide you with any information other than the information contained in this prospectus supplement and the accompanying prospectus, including the documents incorporated by reference herein and therein, and any free writing prospectus we have authorized for use in connection with this offering. We take no responsibility for, and can provide no assurances as to the reliability of, any information that is in addition to or different from that contained or incorporated by reference in this prospectus supplement and the accompanying prospectus. We are not offering to sell these shares in any jurisdiction where the offer or sale is not permitted. You should not assume that the information contained or incorporated by reference in this prospectus supplement or the accompanying prospectus is accurate as of any date other than as of the date of this prospectus supplement or the accompanying prospectus, as the case may be, or in the case of the documents incorporated by reference, the date of such documents, regardless of the time of delivery of this prospectus supplement and the accompanying prospectus or any sale of our shares. Our business, financial condition, liquidity, results of operations and prospects may have changed since those dates.

All references in this prospectus supplement or the accompanying prospectus to "Rocket," the "Company," "we," "us," or "our" mean Rocket Pharmaceuticals, Inc., a Delaware corporation, formerly known as Inotek Pharmaceuticals Corporation, and its subsidiaries, unless we state otherwise or the context otherwise requires.

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

The SEC encourages companies to disclose forward-looking information so that investors can better understand a company's future prospects and make informed investment decisions. This prospectus supplement, the accompanying prospectus and the documents we have filed with the SEC that are incorporated herein and therein by reference contain such forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements, other than statements of historical facts, included or incorporated in this prospectus regarding our strategy, future operations, financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements.

The words "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We cannot guarantee that we actually will achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. There are a number of important factors that could cause our actual results to differ materially from those indicated by these forward-looking statements. These important factors include the factors that we identify in the documents we incorporate by reference in this prospectus supplement and the prospectus, as well as other information we include or incorporate by reference in this prospectus supplement and the prospectus. Many factors could affect our actual results, including those factors described under "Risk Factors" in the definitive proxy statement on Schedule 14A dated December 4, 2017 (the "Merger Proxy") and our Form 10-K for the year ended December 31, 2016, incorporated by reference herein. You should read these factors and other cautionary statements made in this prospectus supplement and the accompanying prospectus and the documents incorporated herein by reference. We do not assume any obligation to update any forward-looking statements made by us. Numerous factors could cause our actual results to differ materially from those described in forward-looking statements, including, among other things:

- The businesses of Inotek Pharmaceuticals Corporation and Rocket Pharmaceuticals, Ltd. may not be integrated successfully or such integration may take longer to accomplish than expected; the expected cost savings and revenue synergies from our reverse merger may not be fully realized within the expected timeframes or at all;
- 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, or the 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.

Please also see the discussion of risks and uncertainties under “Risk Factors” beginning on page S-11, page 3 of the accompanying prospectus, on page 14 of the Merger Proxy, and on page 38 of our most recent Annual Report on Form 10-K, and in our other reports filed with the SEC, incorporated herein by reference.

In light of these assumptions, risks and uncertainties, the results and events discussed in the forward-looking statements contained in this prospectus supplement or the accompanying prospectus or in any document incorporated herein or therein by reference might not occur. Investors are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this prospectus supplement or the accompanying prospectus or the date of the document incorporated by reference herein or therein. We are not under any obligation, and we expressly disclaim any obligation, to update or alter any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law. All subsequent forward-looking statements attributable to us or to any person acting on our behalf are expressly qualified in their entirety by the cautionary statements contained or referred to in this section.

PROSPECTUS SUPPLEMENT SUMMARY

This summary highlights certain information about this offering and selected information contained elsewhere in or incorporated by reference into this prospectus supplement and the accompanying prospectus. This summary is not complete and does not contain all of the information that you should consider before deciding whether to invest in shares of our common stock. For a more complete understanding of our Company and this offering, we encourage you to read and consider carefully the more detailed information in this prospectus supplement and the accompanying prospectus, including the information incorporated by reference into this prospectus supplement and the accompanying prospectus, and the information referred to under the heading "Risk Factors" in this prospectus supplement on page S-11 and on page 3 of the accompanying prospectus, and in the documents incorporated by reference into this prospectus supplement and the accompanying prospectus.

Our Company

We are a multi-platform biotechnology company focused on the development of first-in-class gene therapies for rare and devastating pediatric diseases. We have two lentiviral virus, or LVV, programs currently undergoing clinical testing targeting Fanconi Anemia, which we refer to as FA, a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, and three additional LVV programs targeting other rare genetic diseases. In addition, we have an adeno-associated virus, or AAV, program for which we expect to file an IND application in 2018, which will permit the commencement of human clinical studies thereafter. We have full 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.

Our two leading LVV and AAV technology platforms are each being designed in collaboration with leading academic and industry partners. 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.

For the development of our gene therapy treatments, we are using a modified non-pathogenic virus. 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 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 recent FDA approval of Novartis’s treatment of pediatric acute lymphoblastic leukemia indicates that there is a regulatory pathway forward for gene therapy products.

Pipeline Overview

LVV Programs. Our LVV-based programs utilize third-generation, self-inactivating lentiviral vectors to target selected rare diseases. Currently, we are developing LVV programs to treat FA, Leukocyte Adhesion Deficiency-I, which we refer to as LAD-I, Pyruvate Kinase Deficiency, which we refer to as PKD, and Infantile Malignant Osteopetrosis, which we refer to as IMO. Brief descriptions of these conditions and the Rocket programs for each is set forth below.

Fanconi Anemia (“FA”)

Our LVV-based programs utilize third-generation, self-inactivating lentiviral vectors to correct defects in patients’ hematopoietic stem cells, 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 hematopoietic stem cells 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 AML (acute myeloid leukemia, 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 US/EU is estimated to be about 2,000.

We currently have the following two LVV-based programs targeting FA:

RP-L101. RP-L101 is a program that we in-licensed from Fred Hutchinson Cancer Center in Seattle, Washington, which we refer to as “Hutch.” RP-L101 is currently being studied in a Phase 1 clinical trial that is treating FA patients at Hutch under an IND sponsored by Hutch. We are entitled to the data from this clinical study and hold the commercial rights to the drug being studied under this IND.

RP-L102. RP-L102 is a program that we in-licensed from CIEMAT (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas), which is a leading research institute in Madrid, Spain. RP-L102 is currently being studied in a Phase 1/2 clinical trial treating FA patients with a modified process under an Investigational Medicinal Product Dossier (“IMPD”) 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.

As of September 2017, three patients have received the infusion of gene-corrected stem cells with RP-L101, and four patients have received gene-corrected stem cells with RP-L102. All patients treated as of September 2017 under each protocol have had stable blood counts during the months subsequent to investigational therapy, despite decreases noted during the months and years preceding gene therapy. Improvements in the clinical and cell-processing components of our FA trials may yield more robust and readily-identifiable disease-reversal, for both the RP-L101 and RP-L102 programs.

We expect to announce clinical data from our LVV-based programs targeting FA during the next 12 to 18 months. We expect to advance an LVV-based program targeting FA to the pivotal trial stage in 2019.

Leukocyte Adhesion Deficiency-I ("LAD-I")

LAD-I is a genetic disorder that causes the immune system to malfunction, resulting in a form of immunodeficiency. Immunodeficiencies are conditions in which the immune system is unable to protect the body effectively from foreign invaders such as viruses, bacteria, and fungi. Starting from birth, people with LAD-I frequently develop serious bacterial and fungal infections. Life expectancy in individuals with LAD-I is often severely shortened. Due to repeat infections, affected individuals may not survive past infancy.

We currently have one LVV-based program targeting LAD-I, RP-L201. RP-L201 is a pre-clinical program that Rocket in-licensed from CIEMAT. This program is currently being developed through an ongoing collaboration with CIEMAT, with a rolling IMPD expected to be filed in the fourth quarter of 2018. Upon the filing and clearance of the IMPD, Rocket expects to commence enrolling patients at CIEMAT in a clinical trial.

Pyruvate Kinase Deficiency ("PKD")

PKD is an inherited lack of the enzyme "pyruvate kinase," which is used by red blood cells. Without this enzyme, red blood cells break down too easily, resulting in a low level of these cells, which in turn causes a form of anemia that can range in severity from mild (asymptomatic) to severe (resulting in childhood mortality or the requirement for frequent, lifelong blood transfusions). The pediatric population is the most commonly and severely affected subgroup of patients with PKD, and pediatric patients often undergo splenectomy (removal of the spleen) and experience jaundice and chronic iron overload.

We currently have one LVV-based program targeting PKD, RP-L301. RP-L301 is a pre-clinical program that we in-licensed from CIEMAT. This program is currently being developed through an ongoing collaboration with CIEMAT, with a rolling IMPD expected to be filed in the fourth quarter of 2018. Upon the filing and clearance of the IMPD, we expect to commence enrolling patients at CIEMAT in a clinical trial in the first half of 2019.

Infantile Malignant Osteopetrosis ("IMO")

IMO is a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. Osteopetrosis is a disorder of bone development in which the bones become thickened. Normally, small areas of bone are constantly being broken down by special cells called osteoclasts, then made again by cells called osteoblasts. In osteopetrosis, the cells that break down bone (osteoclasts) do not work properly, which leads to the bones becoming thicker and not as healthy. IMO is a severe form of osteopetrosis that typically presents in the first year of life and is associated with severe manifestations leading to death within the first decade of life without allogeneic hematopoietic stem cell transplantation, a procedure in which a person receives blood-forming stem cells from a genetically similar, but not identical donor. For patients who do receive a bone marrow transplant, results have been limited, with frequent graft failure or rejection (graft-versus-host-disease) and other severe complications. 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.

We currently have one LVV-based program targeting IMO, RP-L401. RP-L401 is a pre-clinical program that Rocket in-licensed from Lund University, Sweden. This program is currently being developed through an ongoing collaboration with Lund University, with an IMPD expected to be filed upon completion of IND/IMPD-enabling studies. Upon the filing and clearance of the IMPD, clinical trials are expected to commence in 2019.

AAV-based Program

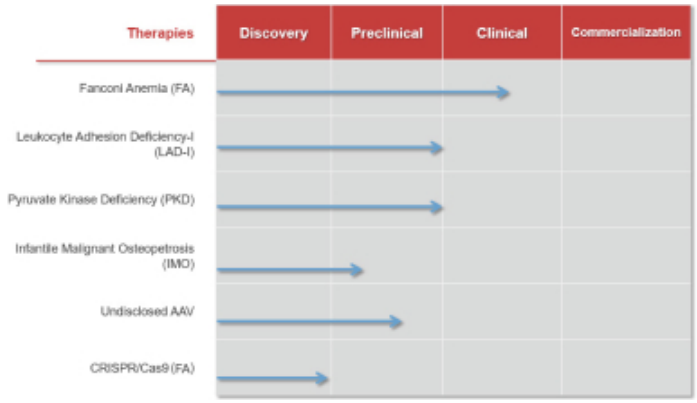
Our AAV-based program involves the direct injection of the viral vector into the patient, rather than modifying the patient's cells ex-vivo. In our preclinical studies of our AAV-based program to date, this method of therapy has displayed substantial tropism, which is the ability to hone in on the organs most afflicted by the underlying disorder, with the aim of modifying cellular function to enable the production of sufficient quantities of a missing protein to restore proper function to the afflicted cells.

We are currently developing RP-A501, which is an AAV-based program for an undisclosed rare disease. This program is currently in pre-clinical development, with IND-enabling studies ongoing. We expect to announce pre-clinical data and the indication for this program in the third quarter of 2018 and to file an IND for this program in the fourth quarter of 2018.

CRISPR/Cas9-based program

In addition to our LVV and AAV programs, we also have 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.

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



Strategy

Our Company seeks 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 target 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 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 expect 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.

Reverse Merger

On September 12, 2017, Inotek Pharmaceuticals Corporation ("Inotek") entered into an Agreement and Plan of Merger and Reorganization with Rocket Pharmaceuticals, Ltd., a privately held Cayman limited company, and Rome Merger Sub, a wholly owned subsidiary of Inotek (the "Merger Subsidiary"), pursuant to which the Merger Subsidiary would be merged with and into Rocket Pharmaceuticals, Ltd. (the "Reverse Merger"), with Rocket Pharmaceuticals, Ltd. continuing after the Merger as the surviving company and a wholly owned subsidiary of Inotek.

On January 4, 2018, in connection with the closing of the Reverse Merger, Inotek effected a reverse stock split at a ratio of one-for-four (the "Reverse Stock Split") and at the effective time of the Reverse Merger, Inotek issued 26,272,107 shares of Inotek's common stock to the former shareholders of Rocket Pharmaceuticals, Ltd. in exchange for all of the issued and outstanding shares of Rocket Pharmaceuticals, Ltd. In connection with the Reverse Merger, Inotek changed its name to "Rocket Pharmaceuticals, Inc." The shares being offered through this prospectus supplement give effect to the Reverse Stock Split.

As a result of the Reverse Stock Split, the initial conversion rate of the combined Company's approximately $52.0 million aggregate principal amount outstanding of 5.75% Convertible Senior Notes due 2021 was automatically adjusted from approximately 125 shares of common stock per $1,000 principal amount of such notes to approximately 31 shares of common stock per $1,000 principal amount of the notes.

We are currently finalizing our financial results for the fiscal year ended December 31, 2017. While complete financial information and operating data are not available, based on information currently available, we estimate that as of the closing of the Reverse Merger on January 4, 2018, our cash, cash equivalents and short-term investments aggregated to approximately $117.2 million. These preliminary estimates have been prepared by, and are the responsibility of, our management. Our independent registered public accounting firms, RSM US LLP and EisnerAmper LLP, have not audited or reviewed, and do not express an opinion with respect to, these estimates. Our actual cash and cash equivalents and short-term investments available for sale as of December 31, 2017 or as of the closing of the Reverse Merger on January 4, 2018 may differ from these estimates due to the completion of our closing procedures, final adjustments and other developments that may arise between now and the time the financial results are finalized.

Corporate Information

We were incorporated under the laws of the State of Delaware under the name "Inotek Pharmaceuticals Corporation" and we changed our name to "Rocket Pharmaceuticals, Inc." on January 4, 2018. We have executive offices located at The Alexandria Center for Life Science, 430 East 29th Street, Suite 1040, New York, NY 10016, 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 SEC. Our SEC reports can be accessed through the Investors section of our website. Further, a copy of our Annual Report on Form 10-K is located at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D. C. 20549. Information on the operation of the Public Reference Room can be obtained by calling the SEC at 1-800-SEC-0330. 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 prospectus supplement or the accompanying prospectus. Our common stock is listed on The Nasdaq Global Market under the symbol "RCKT."

THE OFFERING

Common Stock we are Offering Pursuant to this Prospectus Supplement	shares
Common Stock to be Outstanding after this Offering	shares
Option to Purchase Additional Shares	We have granted the underwriters an option for a period of 30 days from the date of this prospectus supplement to purchase up to additional shares.
Use of Proceeds	We intend to use the net proceeds from this offering to fund the continued development of our pipeline of gene therapies for rare diseases, enhancements to in-house manufacturing, and general corporate purposes. See "Use of Proceeds."
Nasdaq Global Market Symbol	"RCKT"
Risk Factors	This investment involves a high degree of risk. You should read the "Risk Factors" section of this prospectus supplement beginning on page S-11 and page 3 of the accompanying prospectus, and under similar headings in the other documents, including the Merger Proxy, our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q, incorporated by reference into this prospectus supplement and the accompanying prospectus, for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.

The number of shares of common stock shown above to be outstanding after this offering is based on the 33,077,793 shares outstanding as of January 4, 2018 immediately after giving effect to the Reverse Stock Split and the Reverse Merger and excludes:

- 7,482,854 shares of our common stock subject to options outstanding as of January 4, 2018, having a weighted average exercise price of $2.44 per share;
- 271,718 shares of our common stock subject to restricted stock units outstanding as of January 4, 2018;
- 3,424,508 shares of our common stock that have been reserved for issuance in connection with future grants under our 2014 Stock Option and Incentive Plan as of January 4, 2018;
- 14,102 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants as of January 4, 2018, having a weighted average exercise price of $24.816 per share; and
- 1,621,755 shares of common stock issuable upon the conversion of principal due under outstanding 5.75% Convertible Senior Notes due 2021, of which approximately $52.0 million of aggregate principal was outstanding as of January 4, 2018.

If the underwriters' option to purchase additional shares is exercised in full, we will issue and sell an additional shares of our common stock and will have shares outstanding after the offering.

Except as otherwise noted, all information in this prospectus supplement assumes no exercise of the underwriters' option to purchase additional shares.

RISK FACTORS

An investment in our common stock involves a high degree of risk. Before deciding whether to invest in our common stock, you should consider carefully the risks described below and discussed under the sections captioned "Risk Factors" contained in the Merger Proxy, our Annual Report on Form 10-K for the year ended December 31, 2016, and any subsequent Quarterly Reports on Form 10-Q, which are incorporated by reference herein in their entirety, and which may be amended, supplemented or superseded from time to time by other reports we file the SEC in the future, together with other information contained in or incorporated by reference in this prospectus supplement, the accompanying prospectus, and in any free writing prospectus that we have authorized for use in connection with this offering. There may be other unknown or unpredictable economic, business, competitive, regulatory or other factors that could have material adverse effects on our future results. If any of these risks actually occurs, our business, financial condition, results of operations or business prospects could be seriously harmed. This could cause the trading price of our common stock to decline, resulting in a loss of all or part of your investment.

Risks Related To Rocket's Financial Position

Rocket has a history of operating losses, and Rocket may not achieve or sustain profitability. Rocket anticipates that it will continue to incur losses for the foreseeable future. If Rocket fails to obtain additional funding to conduct its planned research and development effort, Rocket could be forced to delay, reduce or eliminate its product development programs or commercial development efforts.

Rocket is 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. Rocket's operations to date have been limited primarily to organizing and staffing its company, business planning, raising capital, acquiring and developing product and technology rights and conducting preclinical research and development activities for its product candidates. Rocket has never generated any revenue from product sales. Rocket has not obtained regulatory approvals for any of its product candidates, and has funded its operations to date through proceeds from sales of its preferred stock.

Rocket has incurred net losses since its inception. Rocket Pharmaceuticals, Ltd. incurred a net loss of $7.6 million for the year ended December 31, 2016, and a net loss of $4.2 million for the period from July 14, 2015 (Rocket Pharmaceuticals, Ltd.'s inception) to December 31, 2015. As of December 31, 2016, Rocket Pharmaceuticals, Ltd. had an accumulated deficit of $11.8 million. Substantially all of its operating losses have resulted from costs incurred in connection with its research and development programs and from general and administrative costs associated with its operations. Rocket expects to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as Rocket intends to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of its product candidates is approved, sales and marketing activities that, together with anticipated general and administrative expenses, will likely result in Rocket incurring significant losses for the foreseeable future. Rocket's prior losses, combined with expected future losses, have had and will continue to have an adverse effect on Rocket's stockholders' deficit and working capital.

Rocket 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 Rocket to delay, limit or terminate certain of its licensing activities, product development efforts or other operations.

Rocket expects to require substantial future capital in order to seek to broaden licensing of its gene therapy platforms, complete preclinical and clinical development for its current product candidates and other future product candidates, if any, and potentially commercialize these product candidates. Rocket expects its spending levels to increase in connection with its preclinical and clinical trials. In addition, if Rocket obtains marketing approval for any of its product candidates, Rocket expects to incur significant expenses related to product sales,

medical affairs, marketing, manufacturing and distribution. Furthermore, Rocket expects to incur additional costs associated with operating as a public company. Accordingly, Rocket will need to obtain substantial additional funding in connection with its continuing operations. If Rocket is unable to raise capital when needed or on acceptable terms, Rocket could be forced to delay, reduce or eliminate certain of its licensing activities, its research and development programs or other operations.

Rocket Pharmaceuticals, Ltd.'s operations have consumed significant amounts of cash since inception. As of December 31, 2016, Rocket Pharmaceuticals, Ltd.'s cash was $9.5 million. As of September 30, 2017 Rocket Pharmaceuticals, Ltd.'s cash was $24.2 million. Rocket's future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement, completion and results of Rocket's clinical trials, including Rocket's only current clinical trial for Fanconi Anemia;
- the results of Rocket's preclinical studies for Rocket's 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 Rocket's internal product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity, if any;
- the costs, timing and outcome of regulatory review of Rocket's product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of Rocket's product candidates for which Rocket receives marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing its intellectual property rights and defending any intellectual property-related claims;
- Rocket's current licensing agreements or collaborations remaining in effect;
- Rocket's ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all;
- the extent to which Rocket acquires or in-licenses other product candidates and technologies; and
- the costs associated with being a public company.

Many of these factors are outside of Rocket's 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 Rocket may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, Rocket's product candidates, if approved, may not achieve commercial success. Accordingly, Rocket will need to continue to rely on additional financing to achieve its 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 Rocket's current shareholders and the terms may include liquidation or other preferences that adversely affect the rights of Rocket's current shareholders. Adequate additional financing may not be available to Rocket on acceptable terms, or at all. Rocket also could be required to seek funds through arrangements with partners or otherwise that may require Rocket to relinquish rights to its intellectual property, its product candidates or otherwise agree to terms unfavorable to Rocket.

Rocket's limited operating history may make it difficult for Rocket to evaluate the success of its business to date and to assess Rocket's future viability.

Rocket's operations to date have predominantly focused on organizing and staffing its company, business planning, raising capital, acquiring its technology, administering and expanding its gene therapy platforms,

identifying potential product candidates, undertaking research, preclinical studies and clinical trials of its product candidates and establishing licensing arrangements and collaborations. Rocket has not yet completed clinical trials of its 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 Rocket's future success or viability may not be as accurate as they could be if Rocket had a longer operating history.

In addition, as a new business, Rocket may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. Rocket expects to eventually transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development activities and Rocket may need to transition to supporting commercial activities in the future. Rocket cannot guarantee that it will be successful in these transitions.

Rocket's ability to use its net operating loss carryforwards and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes to offset its post-change income may be limited. Rocket may experience ownership changes in the future. As a result, if Rocket earns net taxable income, Rocket's ability to use its pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to Rocket. Furthermore, Rocket's ability to use net operating loss carryforwards to offset U.S. federal taxable income in the future may be further limited by certain provisions set forth in The Tax Cuts and Jobs Act, which could potentially result in increased future tax liability to Rocket. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. At December 31, 2016, Rocket Pharmaceuticals, Ltd. had net operating losses of approximately $7.0 million for New York City tax purposes. As of December 31, 2016, Rocket had no unrecognized tax benefits or liabilities for uncertain tax positions. Rocket files income tax returns in the United States and New York State and New York City, but for the year ended December 31, 2016 did not report any income effectively connected with a U.S. trade or business.

Rocket has never generated any revenue from product sales and may never be profitable.

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

- completing research and preclinical and clinical development of Rocket's product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which Rocket completes clinical studies;
- developing a sustainable, commercial-scale, reproducible, and transferable manufacturing process for Rocket's 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 Rocket's product candidates, if approved;
- launching and commercializing product candidates for which Rocket obtains 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 Rocket's product candidates from private and governmental payors;
- obtaining market acceptance of Rocket's 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 Rocket may enter; and
- maintaining, protecting and expanding Rocket's portfolio of intellectual property rights, including patents, trade secrets and know-how.

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

Risks Related To Product Regulatory Matters

Rocket's 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 product has been approved in the United States and the European Union.

Rocket has concentrated its research and development efforts to date on a gene therapy platform, and Rocket's future success depends on the successful development of viable gene therapy product candidates. Rocket cannot guarantee that it 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. Rocket may also experience unanticipated problems or delays in developing Rocket's manufacturing capacity or transferring Rocket's manufacturing process to commercial partners, which may prevent Rocket from completing its clinical studies or commercializing its products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the European Medicines Agency, or 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 Rocket's 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 European Union, including Novartis Pharmaceuticals' Kymriah, Kite Pharma's Yescarta, 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 Rocket's product candidates in the United States, the European Union 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 Rocket's ability to generate sufficient product revenue and Rocket's 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, the FDA's Center for Biologics Evaluation and Research, or CBER, which may require Rocket to perform additional nonclinical studies or clinical trials that may increase Rocket's development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of Rocket's gene therapy product candidates or lead to significant post-approval limitations or restrictions.

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

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

To date, Rocket's experience with clinical trials has been limited. Rocket's only clinical programs to date have been performed under a physician-sponsored investigational new drug application, or IND, held by the Fred Hutchinson Cancer Research Center in Seattle, Washington, or Hutch, and under an Investigational Medicinal Product Dossier, or IMPD, in Spain sponsored by CIEMAT. The clinical trials performed by these sponsors are for a lentiviral treatment for Fanconi Anemia, a rare mutation of the FANC-A gene, which are still ongoing. Rocket intends to assume responsibility for or obtain the authority to reference the clinical trials performed under one or both of the IND and IMPD held by its collaborators, but has not completed any clinical trials to date. Rocket cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial failure can occur at any stage of testing.

Identifying and qualifying patients to participate in clinical trials of Rocket's product candidates is critical to Rocket's success. Rocket 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 Rocket plans to evaluate its 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. 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.

In addition, to the extent Rocket seeks to obtain regulatory approval for its product candidates in foreign countries, Rocket's 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 clinical research organizations, or 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 AAV gene therapy protocols;
- Rocket's inability to locate qualified local partners or collaborators for such clinical trials; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If Rocket has difficulty enrolling a sufficient number of patients to conduct its clinical trials as planned, Rocket 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. Moreover, Rocket intends to rely on the nonclinical studies and clinical trials performed by Hutch and 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 Hutch and CIEMAT studies and trials. Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to Rocket or impair Rocket's ability to generate revenues from product sales, regulatory and commercialization milestones and royalties.

Rocket has not completed any clinical studies of its current product candidates. Initial results in Rocket's 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.

Rocket's Fanconi Anemia gene therapy treatments are currently in clinical trials being conducted by Rocket's partners, Hutch and CIEMAT. Several of Rocket's 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 Rocket's clinical studies or for any future subjects in Rocket's ongoing or future clinical studies, and may not be repeated or observed in ongoing or future studies involving Rocket's product candidates. Furthermore, Rocket's 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. Rocket 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 Rocket's 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 Rocket successfully completes the necessary preclinical studies and clinical trials, Rocket cannot predict when, or if, Rocket will obtain regulatory approval to commercialize a product candidate and the approval may be for a more narrow indication than Rocket seeks.

Rocket cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Rocket has not received approval from regulatory authorities in any jurisdiction to market any of its product candidates. Even if Rocket's 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, Rocket may not be able to obtain regulatory approval. In addition, Rocket may experience delays or rejections if an FDA Advisory Committee recommends disapproval or restrictions on use. In addition, Rocket 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 Rocket's 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 Risk Evaluation and Mitigation Strategies, 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 Rocket's product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for Rocket's product candidates and materially harm its business, financial condition, results of operations and prospects.

Even if Rocket obtains regulatory approval for a product candidate, its products will remain subject to regulatory scrutiny.

Even if Rocket obtains regulatory approval in a jurisdiction, the applicable regulatory authority may still impose significant restrictions on the indicated uses or marketing of Rocket's 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 Biologics License Application, or 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 good manufacturing practices, or GMP, and current good tissue practice, or cGMP, adherence to commitments made in the BLA. If Rocket or a regulatory agency discovers 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 Rocket fails to comply with applicable regulatory requirements following approval of any of its product candidates, a regulatory agency may take a variety of actions, including:

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

Any government investigation of alleged violations of law could require Rocket to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty

described above may inhibit Rocket's ability to commercialize its product candidates and generate revenues and could harm its 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 Rocket's product candidates. Rocket 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 Rocket is slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if Rocket is not able to maintain regulatory compliance, Rocket may lose any marketing approval which Rocket may have obtained and Rocket may not achieve or sustain profitability, which would materially harm Rocket's business, financial condition, results of operations and prospects.

Rocket may never obtain FDA approval for any of its product candidates in the United States, and even if Rocket does, Rocket may never obtain approval for or commercialize any of its product candidates in any other jurisdiction, which would limit Rocket's ability to realize its full market potential.

In order to eventually market any of Rocket's product candidates in any particular foreign jurisdiction, Rocket 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 United States, 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 Rocket 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 Rocket's products in those countries. The foreign regulatory approval process involves similar risks to those associated with FDA approval. Rocket does not have any product candidates approved for sale in any jurisdiction, including international markets, nor has Rocket attempted to obtain such approval. If Rocket fails to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, Rocket's target market will be reduced and Rocket's ability to realize the full market potential of its products will be unrealized.

Rocket's 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 Rocket's 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 Rocket's potential product candidates, Rocket may decide or be required to halt or delay further clinical development of its 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, Rocket's clinical trials could be suspended or terminated. Under certain circumstances, the FDA, the European Commission, the EMA or other regulatory authorities could order Rocket to cease further development of, or deny approval of, Rocket's product candidates for any or all targeted indications. Moreover, if Rocket elects, or

is required, to not initiate or to delay, suspend or terminate any future clinical trial of any of its product candidates, the commercial prospects of such product candidates may be harmed and Rocket's ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm Rocket's ability to develop other product candidates, and may harm Rocket's business, financial condition and prospects significantly.

Furthermore, if undesirable side effects caused by Rocket's 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;
- Rocket may be required to change the way a product candidate is administered or conduct additional clinical trials; and
- Rocket's reputation may suffer.

Any of these occurrences may harm Rocket's business, financial condition and prospects significantly.

Rocket may be unable to obtain orphan drug designation or exclusivity for some product candidates. If Rocket's competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as its product candidates, Rocket may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the U.S. and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the U.S., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the European Union, following the opinion of the EMA's Committee for Orphan Medicinal Products, the European Commission grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before Rocket does (regardless of Rocket's orphan drug designation), Rocket will be precluded from receiving marketing approval for Rocket's product for the applicable exclusivity period. The applicable period is seven years in the U.S. and 10 years in the European Union. The exclusivity period in the U.S. can be extended by six months if the BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if Rocket requests orphan drug designation for any of its product candidates, Rocket cannot guarantee that the FDA or the European Commission will grant any of its product candidates such designation. Additionally, the designation of any of Rocket's product candidates as an orphan product does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as Rocket's product candidates prior to Rocket's product candidates receiving exclusive marketing approval.

Even if Rocket obtains orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. In the U.S., even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

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

Risks Related To Manufacturing, Development and Commercialization Of Rocket's Product Candidates

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

Rocket currently has development, manufacturing and testing agreements with third parties to manufacture supplies of its 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.

Rocket's product candidates require processing steps that are more complex than those required for small molecule pharmaceuticals.

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

Any problems in Rocket's manufacturing process or the facilities with which Rocket contracts could make Rocket a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit Rocket's access to attractive development programs. Problems in third-party manufacturing processes or facilities also could restrict Rocket's ability to meet market demand for Rocket's products. Additionally, should Rocket 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.

Rocket may not successfully commercialize Rocket's drug candidates.

Rocket's gene therapy product candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and Rocket's failure to develop safe, commercially viable

products would severely limit Rocket's ability to become profitable or to achieve significant revenues. Rocket may be unable to successfully commercialize Rocket's product candidates because of several reasons, including:

- some or all of Rocket's product candidates may be found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances;
- Rocket's 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 its product candidates on a scale that is necessary to ultimately deliver its products to end-users;
- proprietary rights of third parties may preclude Rocket from marketing its product candidates; and
- third parties may market superior or equivalent drugs which could adversely affect the commercial viability and success of Rocket's product candidates.

Rocket's ability to successfully develop and commercialize its 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 Rocket's 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 Rocket's products as well as levels at which these payors pay directly for Rocket's products, where applicable, could affect whether Rocket is able to successfully commercialize these products. Rocket cannot guarantee that reimbursement will be available for any of its product candidates. Nor can Rocket guarantee that coverage or reimbursement amounts will not reduce the demand for, or the price of, its product candidates. Rocket has not commenced efforts to have its product candidates reimbursed by government or third-party payors. If coverage and reimbursement are not available or are available only at limited levels, Rocket may not be able to successfully commercialize its 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 European Union, the pricing of prescription drugs is subject to government control. If Rocket's products are or become subject to government regulation that limits or prohibits payment for Rocket's products, or that subjects the price of Rocket's products to governmental control, Rocket may not be able to generate revenue, attain profitability or commercialize its 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 Rocket is unable to obtain adequate levels of reimbursement for its product candidates, Rocket's ability to successfully market and sell its product candidates will be harmed. The manner and level at which reimbursement is provided for services related to Rocket's product candidates (e.g., for administration of Rocket's product to patients) is also important to successful commercialization of its product candidates. Inadequate reimbursement for such services may lead to physician resistance and limit Rocket's ability to market or sell its products.

Rocket faces intense competition and rapid technological change and the possibility that its competitors may develop therapies that are more advanced or effective than Rocket's, which may adversely affect Rocket's financial condition and its ability to successfully commercialize its product candidates.

Rocket is engaged in gene therapy for severe genetic and rare diseases, which is a competitive and rapidly changing field. Rocket has competitors both in the United States and internationally, including major

multinational pharmaceutical companies, biotechnology companies and universities and other research institutions.

Many of Rocket's competitors may have substantially greater financial, technical and other resources, such as larger research and development staff, manufacturing capabilities, experienced marketing and manufacturing organizations. Rocket's 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 Rocket may develop, or achieve earlier patent protection, regulatory approval, product commercialization and market penetration than Rocket. Additionally, technologies developed by Rocket's competitors may render its potential product candidates uneconomical or obsolete, and Rocket may not be successful in marketing Rocket's product candidates against those of Rocket's competitors.

In addition, as a result of the expiration or successful challenge of Rocket's patent rights, Rocket could face increased litigation with respect to the validity and/or scope of patents relating to Rocket's competitors' products. The availability of Rocket's competitors' products could limit the demand, and the price Rocket is able to charge, for any products that Rocket may develop and commercialize, thereby causing harm to Rocket's business, financial condition, results of operations and prospects.

Rocket may not be successful in its efforts to build a pipeline of additional product candidates.

Rocket's business model is centered on applying its 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. Rocket may not be able to continue to identify and develop new product candidates in addition to the pipeline of product candidates that its research and development efforts to date have resulted in. Even if Rocket is successful in continuing to build Rocket's pipeline, the potential product candidates that Rocket identifies may not be suitable for clinical development. If Rocket does not successfully develop and commercialize product candidates based upon its approach, Rocket will not be able to obtain product revenue in future periods, which would likely result in significant harm to Rocket's financial position and results of operations.

The success of Rocket's research and development activities, upon which Rocket primarily focuses, is uncertain.

Rocket's primary focus is on its research and development activities and the clinical testing and commercialization of its product candidates. Research and development was Rocket's most significant operating expense for the year ended December 31, 2016. Research and development activities, 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 Rocket's research and development effort and Rocket's business could ultimately suffer. Rocket anticipates that it will remain principally engaged in research and development activities for an indeterminate, but substantial, period of time.

Risks Related To Third Parties

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

Rocket has relied upon and plans to continue to rely upon third parties, including contract research organizations, which we refer to as CROs, medical institutions, and contract laboratories to monitor and manage

data for Rocket's ongoing preclinical and clinical programs. Nevertheless, Rocket maintains responsibility for ensuring that each of Rocket's clinical trials and preclinical studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and Rocket's reliance on these third parties does not relieve Rocket of its regulatory responsibilities. Rocket and its vendors are required to comply with current requirements on GMP, good clinical practices, or GCP, and good laboratory practice, or GLP, which are a collection of laws and regulations enforced by the FDA, EMA or comparable foreign authorities for all of Rocket's 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 Rocket or any of its vendors fails to comply with applicable regulations, the data generated in Rocket's preclinical studies and clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign authorities may require Rocket to perform additional preclinical studies and clinical trials before approving Rocket's marketing applications. Rocket cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of Rocket's clinical trials comply with GCP regulations. In addition, Rocket's clinical trials must be conducted with products produced consistent with GMP regulations. Rocket's failure to comply with these regulations may require Rocket to repeat clinical trials, which would delay the development and regulatory approval processes.

If any of Rocket's relationships with these third parties, medical institutions, clinical investigators or contract laboratories terminate, Rocket may not be able to enter into arrangements with alternative CROs on commercially reasonable terms, or at all. In addition, Rocket's CROs are not its employees, and except for remedies available to Rocket under its agreements with such CROs, Rocket cannot control whether or not they devote sufficient time and resources to Rocket's ongoing preclinical and clinical programs. If Rocket's 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 Rocket's protocols, regulatory requirements, or for other reasons, Rocket's clinical trials may be extended, delayed or terminated and Rocket may not be able to obtain regulatory approval for or successfully commercialize its product candidates. CROs may also generate higher costs than anticipated. As a result, Rocket's business, financial condition and results of operations and the commercial prospects for Rocket's product candidates could be materially and adversely affected, Rocket's costs could increase, and its 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 occur, which can materially impact Rocket's ability to meet its desired clinical development timelines. Though Rocket carefully manages its relationships with its CROs, Rocket cannot guarantee that Rocket will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on its business, financial condition or results of operations.

Rocket expects to rely on third parties to conduct some or all aspects of its drug product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

Rocket does not expect to independently conduct all aspects of its gene therapy production, product manufacturing, research and preclinical and clinical testing. Rocket currently relies, and expects 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.

Rocket's reliance on these third parties for research and development activities will reduce Rocket's control over these activities but will not relieve Rocket of its 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 Rocket's studies in accordance with regulatory requirements or Rocket's stated study plans and protocols, Rocket 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 its product candidates.

Generally these third parties may terminate their engagements with Rocket at will upon notice. If Rocket needs to enter into alternative arrangements, it could delay Rocket's product development activities.

Reliance on third-party manufacturers entails risks to which Rocket would not be subject if Rocket manufactured the product candidates itself, 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 Rocket's 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 Rocket; and
- disruptions to the operations of its third-party manufacturers or suppliers caused by conditions unrelated to its 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 Rocket's 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.

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

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

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

The commercial success of any of Rocket's product candidates will depend upon its 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 Rocket's products. Even with the requisite approvals from the FDA in the United States, the EMA

in the European Union and other regulatory authorities internationally, the commercial success of Rocket's product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and Rocket's product candidates in particular, as medically beneficial, cost-effective and safe. Any product that Rocket commercializes 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, Rocket may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, Rocket's 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 Rocket's treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission;
- 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 Rocket's 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, 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 Rocket's 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 Rocket's product candidates to achieve market acceptance could materially harm Rocket's business, financial condition, results of operations and prospects.

RTW Investments, LLC, Rocket's principal stockholder, may have the ability to significantly influence all matters submitted to stockholders for approval.

RTW Investments, LLC ("RTW"), in the aggregate, beneficially owns approximately 46.68% 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.

Risks Related To Personnel and Other Risks Related To Rocket's Business

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

Rocket is highly dependent upon the efforts of the company's senior management, including Rocket's Chief Executive Officer, Gaurav Shah, MD; and Rocket's Chief Medical Officer and Head of Development, Jonathan Schwartz, MD; and Rocket's Vice President of Finance, Brian Batchelder. The loss of the services of these individuals and other members of Rocket's senior management could delay or prevent the achievement of research, development, marketing, or product commercialization objectives. Rocket's employment arrangements with the key personnel are "at-will." Rocket does not maintain any "key-man" insurance policies on any of the key employees nor does Rocket intend to obtain such insurance. In addition, due to the specialized scientific nature of Rocket's business, Rocket is highly dependent upon its ability to attract and retain qualified scientific and technical personnel and consultants. In view of the stage of Rocket's organizational development and research and development programs, Rocket has restricted its hiring to research scientists, consultants and a small administrative staff and has made only limited investments in manufacturing, production, sales or regulatory compliance resources. 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 Rocket's operations, however, and Rocket may be unsuccessful in attracting and retaining these personnel.

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

As of December 4, 2017, Rocket had less than 20 full-time employees. As Rocket's business activities expand, Rocket may expand its full-time employee base and hire more consultants and contractors. Rocket's 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. Rocket may not be able to effectively manage the expansion of its operations, which may result in weaknesses in Rocket's infrastructure, operational setbacks, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Rocket's expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If Rocket's management is unable to effectively manage Rocket's growth, Rocket's expenses may increase more than expected, Rocket's ability to generate and/or grow revenues could be reduced and Rocket may not be able to implement its business strategy.

Rocket's 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.

Rocket is exposed to the risk of fraud or other misconduct by its 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 United States and abroad, report financial information or data accurately or disclose unauthorized activities to Rocket. 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 Rocket's reputation or could cause regulatory agencies not to approve Rocket's product candidates. Rocket has 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 Rocket takes to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting Rocket 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 Rocket, and Rocket is not successful in defending the company or asserting its rights, those actions could have a significant impact on Rocket's business, including the imposition of significant fines or other sanctions.

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

Rocket's internal computer systems and those of its current and any future collaborators and other consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While Rocket has not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in Rocket's operations, it could result in a material disruption of Rocket's development programs and its business operations, whether due to a loss of its 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 Rocket's regulatory approval efforts and significantly increase Rocket's 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, Rocket's data or applications, or inappropriate disclosure of confidential or proprietary information, Rocket could incur liability, its competitive position could be harmed and the further development and commercialization of Rocket's product candidates could be delayed.

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

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

Given Rocket's commercial relationships outside of the United States, in particular in the European Union, a variety of risks associated with international operations could harm its business.

Rocket engages in various commercial relationships outside the United States and Rocket may commercialize its product candidates outside of the United States. 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 Rocket, including the Foreign Corrupt Practices Act. Although Rocket may implement policies and procedures specifically designed to comply with these laws and policies, there can be no assurance that Rocket's employees, contractors and agents will comply with these laws and policies. If Rocket is unable to successfully manage the challenges of international expansion and operations, Rocket's business and operating results could be harmed.

Rocket may be, and expect that it will be to the extent Rocket commercializes its product candidates outside the United States, subject to various risks associate 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; and
- greater difficulty with enforcing Rocket's contracts in jurisdictions outside of the United States.

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

Risks Related To Rocket's Intellectual Property

Rocket's rights to intellectual property for the development and commercialization of its product candidates are subject to the terms and conditions of licenses granted to Rocket by others.

Rocket is heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of its technology and products, including technology related to Rocket's manufacturing process and Rocket's 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 Rocket may wish to license its platform or develop or commercialize its technology and products in the future. As a result, Rocket may not be able to prevent competitors from developing and commercializing competitive products in territories not included in all of its licenses.

Licenses to additional third-party technology that may be required for Rocket's 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 Rocket's business and financial condition.

In some circumstances, Rocket 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 Rocket's license from third parties. If Rocket's licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights Rocket has licensed may be reduced or eliminated and Rocket's right to develop and commercialize any of its products that are the subject of such licensed rights could be impacted. In addition to the foregoing, the risks associated with patent rights that Rocket licenses from third parties will also apply to patent rights Rocket may own in the future.

Furthermore, the research resulting in certain of Rocket's 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 Rocket's confidential information to third parties and to exercise march-in rights to use or allow third parties to use Rocket's licensed technology. The government can exercise its march-in rights if it determines that

action is necessary because Rocket fails 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, Rocket's 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 Rocket's competitive position, business, financial condition, results of operations and prospects.

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

Rocket's success depends, in large part, on its ability to obtain and maintain patent protection in the U.S. and other countries with respect to its product candidates and its manufacturing technology. Rocket's licensors have sought and Rocket may intend to seek to protect its proprietary position by filing patent applications in the U.S. and abroad related to many of its novel technologies and product candidates that are important to its business.

The patent prosecution process is expensive, time-consuming and complex, and Rocket 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 Rocket's product candidates have expired or will soon expire. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which Rocket believes precludes its ability to obtain patent protection for certain inventions relating to such work. It is also possible that Rocket will fail to identify patentable aspects of its research and development output before it is too late to obtain patent protection.

Rocket is party to intellectual property license agreements with several entities, each of which is important to its business, and Rocket expects to enter into additional license agreements in the future. Rocket's patent portfolio consists solely of patent applications in-licensed pursuant to those license agreements, and those agreements impose, and Rocket expects that future license agreements will impose, various diligence, development and commercialization timelines, milestone obligations, payments and other obligations on Rocket. If Rocket or its licensees fail to comply with Rocket's obligations under these agreements, or Rocket is subject to a bankruptcy, the licensor may have the right to terminate the license, in which event Rocket could lose certain rights provided by the licenses, including that Rocket may not be able to market products covered by the license. In addition, the patent rights we have in-licensed from Hutch relate only to Hutch's "Prodigy" platform, a portable platform for hematopoietic stem/progenitor cell gene therapy, and not to RP-L101, Rocket's LVV-based program targeting FA that is in-licensed from Hutch.

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 Rocket's patent rights are highly uncertain. Pending and future patent applications may not result in patents being issued which protect Rocket's 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 Rocket's patent rights or narrow the scope of Rocket's 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 2019-2023, if our product were to launch before these dates, we

may need to secure a license. In addition, Rocket may not be aware of all third-party intellectual property rights potentially relating to its 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, Rocket cannot be certain that Rocket was the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that Rocket was the first to file for patent protection of such inventions.

Even if the patent applications Rocket licenses or may own in the future do issue as patents, they may not issue in a form that will provide Rocket with any meaningful protection, prevent competitors or other third parties from competing with Rocket or otherwise provide Rocket with any competitive advantage. Rocket's 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 Rocket's 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 Rocket's 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 Rocket's 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, Rocket's intellectual property may not provide sufficient rights to exclude others from commercializing products similar or identical to Rocket's.

If Rocket breaches its license agreements, it could have a material adverse effect on Rocket's commercialization efforts for its product candidates.

If Rocket breaches any of the agreements under which Rocket licenses intellectual property relating to the use, development and commercialization rights to its product candidates or technology from third parties, Rocket could lose license rights that are important to its business. Licensing of intellectual property is of critical importance to Rocket's business and involves complex legal, business and scientific issues. Disputes may arise between Rocket and its 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 Rocket technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- Rocket's right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- Rocket's diligence obligations with respect to the use of the licensed technology in relation to its 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 Rocket's licensors and Rocket and its partners; and
- whether and the extent to which inventors are able to contest to the assignment of their rights to Rocket's licensors.

If disputes over intellectual property that Rocket has in-licensed prevent or impair Rocket's ability to maintain its current licensing arrangements on acceptable terms, Rocket may be unable to successfully develop and commercialize the affected product candidates. In addition, if disputes arise as to ownership of licensed

intellectual property, Rocket's ability to pursue or enforce the licensed patent rights may be jeopardized. If Rocket or its licensors fail to adequately protect this intellectual property, Rocket's ability to commercialize its products could suffer.

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

If Rocket chooses to engage in legal action to prevent a third-party from using the inventions claimed in its patents or patents which Rocket licenses, 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 Rocket 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 Rocket does 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 Rocket's rights to these patents.

Furthermore, a third-party may claim that Rocket is using inventions covered by the third-party's patent rights and may go to court to stop Rocket from engaging in its normal operations and activities, including making or selling its product candidates. These lawsuits are costly and could affect Rocket's results of operations and divert the attention of managerial and technical personnel. There is a risk that a court would decide that Rocket is infringing the third-party's patents and would order Rocket to stop the activities covered by the patents. In addition, there is a risk that a court will order Rocket 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 Rocket is sued for patent infringement, Rocket would need to demonstrate that its 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. Rocket's competitors have filed, and may in the future file, patent applications covering technology similar to Rocket's. Any such patent application may have priority over Rocket's in-licensed patent applications and could further require Rocket to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to Rocket's, Rocket 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 Rocket's United States patent position with respect to such inventions.

Some of Rocket's competitors may be able to sustain the costs of complex patent litigation more effectively than Rocket 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 Rocket's ability to raise the funds necessary to continue its operations.

If Rocket is unable to protect the confidentiality of its trade secrets, its business and competitive position may be harmed.

In addition to the protection afforded by patents, Rocket relies upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain its competitive position. Rocket seeks to protect its proprietary technology and processes, in part, by entering into confidentiality agreements with its contractors, collaborators, employees and consultants. Nonetheless, Rocket may not be able to prevent the unauthorized disclosure or use of its 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 Rocket does not know whether the

steps Rocket has taken to protect its 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, Rocket may not have adequate remedies for any such breach or violation. As a result, Rocket could lose its trade secrets. Enforcing a claim that a third-party illegally obtained and is using its 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.

Rocket's trade secrets could otherwise become known or be independently discovered by Rocket's competitors. Competitors could purchase Rocket's product candidates and attempt to replicate some or all of the competitive advantages Rocket derives from its development efforts, willfully infringe Rocket's intellectual property rights, design around Rocket's protected technology or develop their own competitive technologies that fall outside of Rocket's intellectual property rights. If any of Rocket's trade secrets were to be lawfully obtained or independently developed by a competitor, Rocket would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with Rocket. If Rocket's trade secrets are not adequately protected or sufficient to provide an advantage over Rocket's competitors, Rocket's competitive position could be adversely affected, as could Rocket's business. Additionally, if the steps taken to maintain Rocket's trade secrets are deemed inadequate, Rocket may have insufficient recourse against third parties for misappropriating Rocket's 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 our patents and patent applications has 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, our patents and patent applications 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. Several patent applications covering our product candidates have been filed recently. 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 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 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 the 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, we 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 our 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 us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

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

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

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

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

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

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

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

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

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid,

is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us 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, or 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. However, 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 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 try 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 employee's former employer 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.

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 have had in the past, and we may also have to

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 have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our 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 employ reputable law firms and other professionals to help us 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 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 involve 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 we might obtain in the future.

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

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

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

Risks Related to this Offering

If you purchase shares in this offering, you will suffer immediate and substantial dilution.

If you purchase shares of our common stock in this offering, you will incur immediate and substantial dilution in the as adjusted net tangible book value of your stock because the price that you pay will be substantially greater than the net tangible book value per share of the shares you acquire. To the extent we raise additional capital by issuing equity securities, our stockholders will experience substantial additional dilution. For a further description of the dilution that you will experience immediately after this offering, see the section of this prospectus supplement titled “Dilution.”

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our business, financial condition or results of operations or

enhance the value of our common stock. The net proceeds from this offering are intended to be used to fund the continued development of our pipeline of gene therapies for rare diseases, enhancements to in-house manufacturing, and general corporate purposes. We may also use a portion of the net proceeds to in-license, acquire or invest in complementary businesses or products.

The failure by our management to apply these funds effectively could result in financial losses that could harm our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

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

We have never declared or paid cash dividends on shares of our common stock. We anticipate that we will retain our earnings, if any, for future growth and therefore do not anticipate paying cash dividends in the future. As a result, only appreciation of the price of our shares of common stock will provide a return to shareholders.

Sales of a significant number of shares of our common stock in the public markets, or the perception that such sales could occur, could depress the market price of our common stock.

Sales of a substantial number of our shares in the public markets could depress the market price of our common stock and impair our ability to raise capital through the sale of additional equity securities. We, our directors and our executive officers and certain of our stockholders have agreed not to sell, dispose of or hedge any shares or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus supplement continuing through and including the date 90 days after the date of this prospectus supplement, subject to certain exceptions. The underwriters may, in their discretion, release the restrictions on any such shares at any time without notice. We cannot predict the effect that future sales of our common stock would have on the market price of our common stock.

USE OF PROCEEDS

We estimate that the net proceeds from the sale of the shares of common stock that we are offering will be approximately $ million, or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares of common stock, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds from this offering to fund the continued development of our pipeline of gene therapies for rare diseases, enhancements to in-house manufacturing, and general corporate purposes.

Pending the application of the net proceeds as set forth above, we intend to invest the net proceeds in short-term, interest-bearing, investment-grade securities.

DILUTION

Rocket Pharmaceuticals, Ltd.'s unaudited historical net tangible book value as of September 30, 2017 was $21.8 million, or $244.58 per share. Inotek's unaudited historical net tangible book value as of September 30, 2017 was $51.1 million, or $1.88 per share. Such historical net tangible book value per share represents the total amount of our tangible assets reduced by the total amount of our liabilities divided by total outstanding common shares (with respect to Inotek) or total outstanding ordinary shares (with respect to Rocket Pharmaceuticals, Ltd.), as of September 30, 2017.

However, as a result of the Reverse Stock Split and the Reverse Merger, the Company believes that an appropriate presentation of the Company's net tangible book value and any dilution per share resulting from this offering should include pro forma adjustments to reflect the Reverse Stock Split and the Reverse Merger. The Company's pro forma as adjusted net tangible book value as of September 30, 2017, after assuming the effectiveness of the Reverse Stock Split and the Reverse Merger, was $75.2 million, or $2.27 per share, based on 33,077,793 shares of our common stock outstanding as of January 4, 2018 immediately after giving effect to the Reverse Stock Split and the Reverse Merger. As this section is presented on a pro forma basis as of September 30, 2017 to reflect the Reverse Stock Split and the Reverse Merger, effective January 4, 2018, you should read this section together with the section titled "Unaudited Pro Forma Combined Financial Statements," which appears in the Merger Proxy beginning on page 131 and which is incorporated by reference in this prospectus.

The Company's pro forma as adjusted unaudited net tangible book value as of September 30, 2017, after giving effect to the Reverse Stock Split and the Reverse Merger and the issuance and sale by us of shares in this offering, would have been $ million, or $ per share based on (i) 33,077,793 shares of our common stock outstanding as of January 4, 2018 and (ii) shares issued in this offering.

Based on the public offering price of $ per share, this represents an immediate increase in the pro forma net tangible book value of $ per share to shareholders and an immediate dilution of $ per share to new investors purchasing our shares in this offering.

Dilution per share represents the difference between the price per share to be paid for the shares sold by us in this offering and the pro forma as adjusted net tangible book value per share after giving effect to the Reverse Stock Split and the Reverse Merger. The following table illustrates this per share dilution for purchasers of common stock in this offering:

Public offering price per share		$
As adjusted pro forma net tangible book value per share as of September 30, 2017 after giving effect to the Reverse Stock Split and the Reverse Merger	$2.27	
Increase in net tangible book value per share attributable to new investors		
Pro forma as adjusted net tangible book value per share after the Reverse Stock Split, the Reverse Merger and this offering		
Dilution per share to new investors in this offering		$

If the underwriters exercise their option to purchase additional shares in full, and based on a public offering price of $ per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, the pro forma net tangible book value per share after giving effect to the Reverse Stock Split, the Reverse Merger and this offering as of September 30, 2017 would be approximately $ per share, the increase in the pro forma net tangible book value per share after giving effect to the Reverse Merger and this offering attributable to new investors would be approximately $ per share and the dilution to new investors purchasing shares in this offering would be approximately $ per share.

The number of shares of common stock shown above to be outstanding after this offering is based on the 33,077,793 shares outstanding as of January 4, 2018 immediately after giving effect to the Reverse Stock Split and the Reverse Merger and excludes:

- 7,482,854 shares of our common stock subject to options outstanding as of January 4, 2018, having a weighted average exercise price of $2.44 per share;
- 271,718 shares of our common stock subject to restricted stock units outstanding as of January 4, 2018;
- 3,424,508 shares of our common stock that have been reserved for issuance in connection with future grants under our 2014 Stock Option and Incentive Plan as of January 4, 2018;
- 14,102 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants as of January 4, 2018, having a weighted average exercise price of $24.816 per share; and
- 1,621,755 shares of common stock issuable upon the conversion of outstanding principal due under outstanding 5.75% Convertible Senior Notes due 2021, of which approximately $52.0 million of aggregate principal was outstanding as of January 4, 2018.

DIVIDEND POLICY

We have never declared or paid any cash dividends on our common 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 any future indebtedness that we may incur could preclude us from paying cash dividends.

UNDERWRITING

We and the underwriters for the offering named below have entered into an underwriting agreement with respect to the common stock being offered. Subject to the terms and conditions of the underwriting agreement, each underwriter has severally agreed to purchase from us the number of shares of our common stock set forth opposite its name below. Cowen and Company, LLC and Evercore Group L.L.C. are the representatives of the underwriters.

Underwriter	Number of Shares
Cowen and Company, LLC	
Evercore Group L.L.C.	
Total	

The underwriting agreement provides that the obligations of the underwriters are subject to certain conditions precedent and that the underwriters have agreed, severally and not jointly, to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased, other than those shares covered by the option to purchase additional shares described below. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the non-defaulting underwriters may be increased or the underwriting agreement may be terminated.

We have agreed to indemnify the underwriters against specified liabilities, including liabilities under the Securities Act of 1933, as amended, and to contribute to payments the underwriters may be required to make in respect thereof.

The underwriters are offering the shares, subject to prior sale, when, as and if issued to and accepted by them, subject to approval of legal matters by their counsel and other conditions specified in the underwriting agreement. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Option to Purchase Additional Shares. We have granted to the underwriters an option to purchase up to additional shares of common stock at the public offering price, less the underwriting discount. This option is exercisable for a period of 30 days. To the extent that the underwriters exercise this option, the underwriters will purchase additional shares from us in approximately the same proportion as shown in the table above.

Discounts and Commissions. The following table shows the public offering price, underwriting discount and proceeds, before expenses to us. These amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

We estimate that the total expenses of the offering, excluding underwriting discount, will be approximately $ and are payable by us. We also have agreed to reimburse the underwriters for up to $15,000 for their FINRA counsel fee. In accordance with FINRA Rule 5110, this reimbursed fee is deemed underwriting compensation for this offering.

		Total	
	Per Share	Without Option	With Option
Public offering price			
Underwriting discount			
Proceeds, before expenses, to Company			

The underwriters propose to offer the shares of common stock to the public at the public offering price set forth on the cover of this prospectus. The underwriters may offer the shares of common stock to securities dealers at the public offering price less a concession not in excess of $ per share. If all of the shares are not sold at the public offering price, the underwriters may change the offering price and other selling terms. Sales of shares of common stock may be made by affiliates of certain of the underwriters.

Discretionary Accounts. The underwriters do not intend to confirm sales of the shares to any accounts over which they have discretionary authority.

Stabilization. In connection with this offering, the underwriters may engage in stabilizing transactions, overallotment transactions, syndicate covering transactions, penalty bids and purchases to cover positions created by short sales.

- Stabilizing transactions permit bids to purchase shares of common stock so long as the stabilizing bids do not exceed a specified maximum, and are engaged in for the purpose of preventing or retarding a decline in the market price of the common stock while the offering is in progress.
- Overallotment transactions involve sales by the underwriters of shares of common stock in excess of the number of shares the underwriters are obligated to purchase. This creates a syndicate short position which may be either a covered short position or a naked short position. In a covered short position, the number of shares over-allotted by the underwriters is not greater than the number of shares that they may purchase in the overallotment option. In a naked short position, the number of shares involved is greater than the number of shares in the overallotment option. The underwriters may close out any short position by exercising their option to purchase additional shares and/or purchasing shares in the open market.
- Syndicate covering transactions involve purchases of common stock in the open market after the distribution has been completed in order to cover syndicate short positions. In determining the source of shares to close out the short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared with the price at which they may purchase shares through exercise of the option to purchase additional shares. If the underwriters sell more shares than could be covered by exercise of the option to purchase additional shares and, therefore, have a naked short position, the position can be closed out only by buying shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that after pricing there could be downward pressure on the price of the shares in the open market that could adversely affect investors who purchase in the offering.
- Penalty bids permit the representatives to reclaim a selling concession from a syndicate member when the common stock originally sold by that syndicate member is purchased in stabilizing or syndicate covering transactions to cover syndicate short positions.

These stabilizing transactions, syndicate covering transactions and penalty bids may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock in the open market may be higher than it would otherwise be in the absence of these transactions. Neither we nor the underwriters make any representation or prediction as to the effect that the transactions described above may have on the price of our common stock. These transactions may be effected on The Nasdaq Stock Market, in the over-the-counter market or otherwise and, if commenced, may be discontinued at any time.

Passive Market Making. In connection with this offering, underwriters and selling group members may engage in passive market making transactions in our common stock on The Nasdaq Stock Market in accordance with Rule 103 of Regulation M under the Securities Exchange Act of 1934, as amended, during a period before the commencement of offers or sales of common stock and extending through the completion of the distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker’s bid, such bid must then be lowered when specified purchase limits are exceeded.

Lock-Up Agreements. Pursuant to certain “lock-up” agreements, we and our executive officers, directors and certain of our stockholders, have agreed, subject to certain exceptions, not to offer, sell, assign, transfer, pledge, contract to sell, or otherwise dispose of or announce the intention to otherwise dispose of, or enter into any swap, short sale, hedge or similar agreement or arrangement that transfers, in whole or in part, any of the economic

consequence of ownership of, directly or indirectly, or make any demand or request or exercise any right with respect to the registration of, or file with the SEC a registration statement under the Securities Act relating to, any common stock or securities convertible into or exchangeable or exercisable for any common stock without the prior written consent of Cowen and Company, LLC and Evercore Group L.L.C., for a period of 90 days after the date of the underwriting agreement.

Cowen and Company, LLC and Evercore Group L.L.C. in their sole discretion, may release our common stock and other securities subject to the lock-up agreements described above in whole or in part at any time.

Canada. The common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration Requirements, Exemptions and Ongoing Registrant Obligations*. Any resale of the common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus supplement (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 *Underwriting Conflicts* (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

United Kingdom. Each of the underwriters has represented and agreed that:

- it has not made or will not make an offer of the securities to the public in the United Kingdom within the meaning of section 102B of the Financial Services and Markets Act 2000 (as amended) (FSMA) except to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities or otherwise in circumstances which do not require the publication by us of a prospectus pursuant to the Prospectus Rules of the Financial Services Authority (FSA);
- it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of section 21 of FSMA) to persons who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 or in circumstances in which section 21 of FSMA does not apply to us; and
- it has complied with and will comply with all applicable provisions of FSMA with respect to anything done by it in relation to the securities in, from or otherwise involving the United Kingdom.

Switzerland. The securities will not be offered, directly or indirectly, to the public in Switzerland and this prospectus does not constitute a public offering prospectus as that term is understood pursuant to article 652a or 1156 of the Swiss Federal Code of Obligations.

European Economic Area. In relation to each Member State of the European Economic Area (the "EEA") which has implemented the European Prospectus Directive (each, a "Relevant Member State"), an offer of our shares may not be made to the public in a Relevant Member State other than:

- to any legal entity which is a qualified investor, as defined in the European Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the European Prospectus Directive), subject to obtaining the prior consent of the relevant dealer or dealers nominated by us for any such offer; or
- in any other circumstances falling within Article 3(2) of the European Prospectus Directive,

provided that no such offer of our shares shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the European Prospectus Directive or supplement prospectus pursuant to Article 16 of the European Prospectus Directive.

For the purposes of this description, the expression an "offer to the public" in relation to the securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the expression may be varied in that Relevant Member State by any measure implementing the European Prospectus Directive in that member state, and the expression "European Prospectus Directive" means Directive 2003/71/EC (and amendments hereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State) and includes any relevant implementing measure in each Relevant Member State. The expression 2010 PD Amending Directive means Directive 2010/73/EU.

Israel. In the State of Israel this prospectus shall not be regarded as an offer to the public to purchase shares of common stock under the Israeli Securities Law, 5728–1968, which requires a prospectus to be published and authorized by the Israel Securities Authority, if it complies with certain provisions of Section 15 of the Israeli Securities Law, 5728–1968, including, inter alia, if: (i) the offer is made, distributed or directed to not more than 35 investors, subject to certain conditions (the "Addressed Investors"); or (ii) the offer is made, distributed or directed to certain qualified investors defined in the First Addendum of the Israeli Securities Law, 5728–1968, subject to certain conditions (the "Qualified Investors"). The Qualified Investors shall not be taken into account in the count of the Addressed Investors and may be offered to purchase securities in addition to the 35 Addressed Investors. The company has not and will not take any action that would require it to publish a prospectus in accordance with and subject to the Israeli Securities Law, 5728–1968. We have not and will not distribute this prospectus or make, distribute or direct an offer to subscribe for our common stock to any person within the State of Israel, other than to Qualified Investors and up to 35 Addressed Investors.

Qualified Investors may have to submit written evidence that they meet the definitions set out in of the First Addendum to the Israeli Securities Law, 5728–1968. In particular, we may request, as a condition to be offered common stock, that Qualified Investors will each represent, warrant and certify to us and/or to anyone acting on our behalf: (i) that it is an investor falling within one of the categories listed in the First Addendum to the Israeli Securities Law, 5728–1968; (ii) which of the categories listed in the First Addendum to the Israeli Securities Law, 5728–1968 regarding Qualified Investors is applicable to it; (iii) that it will abide by all provisions set forth in the Israeli Securities Law, 5728–1968 and the regulations promulgated thereunder in connection with the offer to be issued common stock; (iv) that the shares of common stock that it will be issued are, subject to exemptions available under the Israeli Securities Law, 5728–1968: (a) for its own account; (b) for investment purposes only; and (c) not issued with a view to resale within the State of Israel, other than in accordance with the provisions of the Israeli Securities Law, 5728–1968; and (v) that it is willing to provide further evidence of its Qualified Investor status. Addressed Investors may have to submit written evidence in respect of their identity and may have to sign and submit a declaration containing, inter alia, the Addressed Investor's name, address and passport number or Israeli identification number.

We have not authorized and do not authorize the making of any offer of securities through any financial intermediary on our behalf, other than offers made by the underwriters and their respective affiliates, with a view to the final placement of the securities as contemplated in this document. Accordingly, no purchaser of the shares, other than the underwriters, is authorized to make any further offer of shares on our behalf or on behalf of the underwriters.

Electronic Offer, Sale and Distribution of Shares. A prospectus in electronic format may be made available on the websites maintained by one or more of the underwriters or selling group members, if any, participating in this offering and one or more of the underwriters participating in this offering may distribute prospectuses electronically. The representatives may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the underwriters and selling group members that will make internet distributions on the same basis as other allocations. Other than the prospectus in electronic format, the information on these websites is not part of this prospectus or the registration statement of which this prospectus forms a part, has not been approved or endorsed by us or any underwriter in its capacity as underwriter, and should not be relied upon by investors.

Other Relationships. Certain of the underwriters and their affiliates have provided, and may in the future provide, various investment banking, commercial banking and other financial services for us and our affiliates for which they have received, and may in the future receive, customary fees.

LEGAL MATTERS

Gibson, Dunn & Crutcher LLP, San Francisco, California, will pass upon the validity of the shares of common stock offered hereby. Certain legal matters will be passed upon for the underwriters by Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C., Boston, Massachusetts.

EXPERTS

Inotek Pharmaceuticals Corporation

The consolidated financial statements of Inotek Pharmaceuticals Corporation as of December 31, 2016 and 2015 and for each of the years in the two year period ended December 31, 2016 incorporated in this prospectus supplement by reference from the Inotek Pharmaceuticals Corporation Annual Report on Form 10-K for the year ended December 31, 2016 have been audited by RSM US LLP, an independent registered public accounting firm, as stated in their report thereon, incorporated herein by reference, and have been incorporated in this prospectus supplement in reliance upon such report and upon the authority of such firm as experts in accounting and auditing.

Rocket Pharmaceuticals, Ltd.

The balance sheets of Rocket Pharmaceuticals, Ltd. as of December 31, 2016 and 2015, and the related statements of operations, stockholders' equity, and cash flows for the year ended December 31, 2016 and the period July 14, 2015 (inception) to December 31, 2015, have been audited by EisnerAmper LLP, an independent registered public accounting firm, as stated in their report which is incorporated herein by reference. Such financial statements have been incorporated herein by reference in reliance on the report of such firm given upon their authority as experts in accounting and auditing.

INFORMATION INCORPORATED BY REFERENCE

The SEC allows the Company to "incorporate by reference" the information that is filed by the Company with the SEC, which means that the Company can disclose important information to you by referring you to those documents. The documents incorporated by reference are:

- The Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2016, filed with the SEC on March 16, 2017;
- The Company's Quarterly Reports on Form 10-Q for the quarters ended March 31, 2017, June 30, 2017 and September 30, 2017, filed with the SEC on May 10, 2017, August 3, 2017 and November 8, 2017, respectively;
- The Company's Current Reports on Form 8-K filed with the SEC on January 3, 2017, April 13, 2017, July 7, 2017, August 8, 2017, September 1, 2017, September 13, 2017, October 12, 2017, December 22, 2017 and January 5, 2018;
- The Company's Definitive Proxy Statement on Schedule 14A, filed on each of April 26, 2017 and December 4, 2017;
- All of our filings pursuant to the Exchange Act after the date of filing this prospectus supplement and prior to completion of the offering of securities being made hereby; and
- The description of our common stock contained in our registration statement on Form 8-A, which was filed with the SEC on February 2, 2015, and amended on January 11, 2018, including any amendment or report filed for the purpose of updating such description.

All documents we file with the SEC pursuant to Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act, except as to any portion of any report or documents that is not deemed filed under such provisions, on or after the date of this prospectus supplement until the earlier of the date on which all of the securities registered hereunder have been sold or the registration statement of which this prospectus supplement is a part has been withdrawn, shall be deemed incorporated by reference in this prospectus supplement and to be a part of this prospectus supplement from the date of filing of those documents.

Any statement in this prospectus supplement or the accompanying prospectus or in a document incorporated or deemed to be incorporated by reference into this prospectus supplement or the accompanying prospectus shall be deemed to be modified or superseded, for purposes of this prospectus supplement or the accompanying prospectus, to the extent that a statement contained in or omitted from this prospectus supplement or the accompanying prospectus, or in any other subsequently filed document that also is or is deemed to be incorporated by reference into this prospectus supplement and the accompanying prospectus, modifies or supersedes such statement. Any such statement so modified or superseded shall not be deemed, except as so modified or superseded, to constitute a part of this prospectus supplement and the accompanying prospectus. Nothing in this prospectus supplement shall be deemed to incorporate information furnished but not filed with the SEC pursuant to Item 2.02 or Item 7.01 of Form 8-K.

Upon written or oral request, we will provide, without charge, to each person, including any beneficial owner, to whom a copy of this prospectus supplement is delivered, upon such person's written or oral request, a copy of any and all of the information incorporated by reference in this prospectus supplement, other than exhibits to such documents, unless such exhibits are specifically incorporated by reference into the information that this prospectus supplement incorporates. Requests should be directed to the Secretary at Rocket Pharmaceuticals, Inc. The Alexandria Center for Life Science, 430 East 29th Street, Suite 1040, New York, NY 10016, telephone number (646) 440-9100. You may also find these documents in the "Investor Relations" section of our website, *www.rocketpharma.com/investors-media/*. The information on our website is not incorporated into this prospectus supplement or the accompanying prospectus. We have authorized no one to provide you with any information that differs from that contained in this prospectus supplement and the accompanying prospectus.

Accordingly, you should not rely on any information that is not contained in this prospectus supplement or the accompanying prospectus. You should not assume that the information in this prospectus is accurate as of any date other than the date of the front cover of this prospectus supplement.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

The Company is subject to the informational requirements of the Exchange Act, and in accordance therewith, files annual, quarterly and special reports, proxy statements and other information with the SEC. You may read and copy any document filed by the Company at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the Public Reference Room. The Company's filings with the SEC are also available to the public at the SEC's Internet website at *http://www.sec.gov*. Statements contained in this prospectus supplement as to the contents of any contract or other document are not necessarily complete, and in each instance we refer you to the copy of the contract or document filed as an exhibit to the registration statement, each such statement being qualified in all respects by such reference.

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PROSPECTUS

Inotek Pharmaceuticals Corporation

$200,000,000

Common Stock

Preferred Stock

Debt Securities

Warrants

Units

From time to time, we may offer, issue and sell up to $200,000,000 of any combination of the securities described in this prospectus in one or more offerings. We may also offer securities as may be issuable upon conversion, redemption, repurchase, exchange or exercise of any securities registered hereunder, including any applicable antidilution provisions.

This prospectus provides a general description of the securities we may offer. Each time we offer securities, we will provide specific terms of the securities offered in a supplement to this prospectus. We may also authorize one or more free writing prospectuses to be provided to you in connection with these offerings. The prospectus supplement and any related free writing prospectus may also add, update or change information contained in this prospectus. You should carefully read this prospectus, the applicable prospectus supplement and any related free writing prospectus, as well as any documents incorporated by reference, before you invest in any of the securities being offered.

This prospectus may not be used to sell our securities unless accompanied by a prospectus supplement. The prospectus supplement or any related free writing prospectus may also add to, update, supplement or clarify information contained in this prospectus.

Our common stock is traded on The NASDAQ Global Market under the symbol “ITEK”. The last reported sales price of our common stock on The NASDAQ Global Market on April 1, 2016 was $7.63 per share.

We may offer and sell our securities to or through one or more agents, underwriters, dealers or other third parties or directly to one or more purchasers on a continuous or delayed basis. If agents, underwriters or dealers are used to sell our securities, we will name them and describe their compensation in a prospectus supplement. The price to the public of our securities and the net proceeds we expect to receive from the sale of such securities will also be set forth in a prospectus supplement.

Investing in our securities involves a high degree of risk. You should review carefully the risks and uncertainties referenced under the heading “Risk Factors” on page 3 of this prospectus as well as those contained in the applicable prospectus supplement and any related free writing prospectus, and in the other documents that are incorporated by reference into this prospectus or the applicable prospectus supplement.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The date of this prospectus is April 14, 2016.

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We are responsible for the information contained and incorporated by reference in this prospectus, in any accompanying prospectus supplement, and in any related free writing prospectus we prepare or authorize. We have not authorized anyone to give you any other information, and we take no responsibility for any other information that others may give you. If you are in a jurisdiction where offers to sell, or solicitations of offers to purchase, the securities offered by this documentation are unlawful, or if you are a person to whom it is unlawful to direct these types of activities, then the offer presented in this document does not extend to you. The information contained in this document speaks only as of the date of this document, unless the information specifically indicates that another date applies. Our business, financial condition, results of operations and prospects may have changed since those dates.

ABOUT THIS PROSPECTUS

This prospectus provides you with a general description of our securities being offered. You should read this prospectus together with the additional information described under the heading “Additional Information” and “Incorporation of Certain Information by Reference.”

Under this shelf registration, we may offer shares of our common stock and preferred stock, various series of warrants to purchase common stock or preferred stock, debt securities or any combination thereof, from time to time in one or more offerings. This prospectus only provides you with a general description of the securities we may offer. Each time we offer a type or series of securities under this prospectus, we will provide a prospectus supplement that will contain more specific information about the specific terms of the offering. We may also authorize one or more free writing prospectuses to be provided to you that may contain material information relating to these offerings. This prospectus may not be used to sell our securities unless accompanied by a prospectus supplement. Each such prospectus supplement and any free writing prospectus that we may authorize to be provided to you may also add, update or change information contained in this prospectus or in documents incorporated by reference into this prospectus. We urge you to carefully read this prospectus, any applicable prospectus supplement and any related free writing prospectus, together with the information incorporated herein by reference as described under the headings “Where You Can Find Additional Information” and “Incorporation of Certain Information by Reference” before you invest in our securities.

We have not authorized anyone to provide you with information in addition to or different from that contained in this prospectus, any applicable prospectus supplement and any related free writing prospectus. We take no responsibility for, and can provide no assurances as to the reliability of, any information not contained in this prospectus, any applicable prospectus supplement or any related free writing prospectus that we may authorize to be provided to you. This prospectus is an offer to sell only the securities offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so. You should assume that the information in this prospectus, any applicable prospectus supplement or any related free writing prospectus is accurate only as of the date on the front of the document and that any information incorporated by reference is accurate only as of the date of the document incorporated by reference, regardless of the time of delivery of this prospectus, any applicable prospectus supplement or any related free writing prospectus, or any sale of a security.

This prospectus contains summaries of certain provisions contained in some of the documents described herein, but reference is made to the actual documents for complete information. All of the summaries are qualified in their entirety by the actual documents. Copies of some of the documents referred to herein have been filed, will be filed or will be incorporated by reference as exhibits to the registration statement of which this prospectus is a part, and you may obtain copies of those documents as described below under the heading “Where You Can Find Additional Information”.

Unless otherwise mentioned or unless the context requires otherwise, throughout this prospectus, any applicable prospectus supplement and any related free writing prospectus, the words “Inotek”, “we”, “us”, “our”, the “company” or similar references refer to Inotek Pharmaceuticals Corporation and its subsidiaries; and the term “securities” refers collectively to our common stock, preferred stock, warrants to purchase common stock or preferred stock, debt securities, or any combination of the foregoing securities.

We own various U.S. federal trademark registrations and applications and unregistered trademarks, including our corporate logo. This prospectus and the information incorporated herein by reference contains references to trademarks, service marks and trade names owned by us or other companies. Solely for convenience, trademarks, service marks and trade names referred to in this prospectus and the information incorporated herein, including logos, artwork, and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks, service marks and trade names. We do not intend our use or display of other companies’ trade names, service marks or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies. All trademarks, service marks and trade names included or incorporated by reference into this prospectus, any applicable prospectus supplement or any related free writing prospectus are the property of their respective owners.

COMPANY OVERVIEW

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of therapies for glaucoma and other diseases of the eye. Glaucoma is a disease of the eye that is typically characterized by structural evidence of optic nerve damage, vision loss and consistently elevated intraocular pressure, or IOP. Our lead product candidate, *trabodenoson*, is a first-in-class selective adenosine mimetic that we rationally designed to lower IOP by restoring the eye's natural pressure control mechanism. We developed this molecule to selectively stimulate a particular adenosine subreceptor in the eye with the effect of augmenting the intrinsic function of the eye's trabecular meshwork, or TM. The TM regulates the pressure inside the eye and is also the main outflow path for the fluid inside of the eye that often builds up pressure in patients with glaucoma. We believe that by restoring the natural function of the TM and this outflow path, rather than changing the fundamental dynamics of pressure regulation in the eye, *trabodenoson*'s mechanism of action should result in a lower risk of unintended side effects and long term safety issues than other mechanisms of action. Additionally, *trabodenoson*'s unique mechanism of action in the TM should complement the activity of existing glaucoma therapies that exert their IOP-lowering effects on different parts of the in-flow and out-flow system of the eye. Our product pipeline includes *trabodenoson* monotherapy delivered in an eye drop formulation, as well as a fixed-dose combination, or FDC, of *trabodenoson* with *latanoprost* given once-daily, or QD. We are also evaluating the potential of *trabodenoson* to slow the loss of vision associated with glaucoma and degenerative retinal diseases.

CORPORATE INFORMATION

We were incorporated under the laws of the state of Delaware in 1999. Our principal executive office is located at 91 Hartwell Avenue, Lexington, Massachusetts, 02421, and our telephone number is (781) 676-2100. Our website address is *www.inotekpharma.com*. The information on, or that can be accessed through, our website does not constitute part of this prospectus, and you should not rely on any such information in making the decision whether to purchase our common stock. Our common stock trades on The NASDAQ Global Market under the symbol "ITEK".

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of our initial public offering in February 2015, (b) in which we have total annual gross revenue of at least $1.0 billion, or (c) in which we are deemed to be a large accelerated filer, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. References herein to "emerging growth company" shall have the meaning associated with it in the JOBS Act.

RISK FACTORS

Investing in our securities involves a high degree of risk. You should carefully consider the risks and uncertainties described in the documents incorporated by reference in this prospectus and any prospectus supplement, as well as other information we include or incorporate by reference into this prospectus and any applicable prospectus supplement, before making an investment decision. Our business, financial condition or results of operations could be materially adversely affected by the materialization of any of these risks. The trading price of our securities could decline due to the materialization of any of these risks, and you may lose all or part of your investment. This prospectus and the documents incorporated herein by reference also contain forward-looking statements that involve risks and uncertainties. Actual results could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including the risks described in the documents incorporated herein by reference, including our most recent Annual Report on Form 10-K for the year ended December 31, 2015, as amended, which is on file with the SEC and is incorporated by reference into this prospectus, and other documents we file with the SEC that are deemed incorporated by reference into this prospectus.

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the documents that we incorporate by reference, contains forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act. Any statements about our expectations, beliefs, plans, objectives, assumptions or future events or performance are not historical facts and may be forward-looking. These statements are often, but are not always, made through the use of words or phrases such as “may”, “will”, “could”, “should”, “expects”, “intends”, “plans”, “anticipates”, “believes”, “estimates”, “predicts”, “projects”, “potential”, “continue”, and similar expressions, or the negative of these terms, or similar expressions. Accordingly, these statements involve estimates, assumptions and uncertainties which could cause actual results to differ materially from those expressed in them. Any forward-looking statements are qualified in their entirety by reference to the factors discussed throughout this prospectus, and in particular those factors referenced in the section “Risk Factors.”

This prospectus, including the sections entitled “About this Prospectus” and “Risk Factors,” contains forward-looking statements that are based on our management’s belief and assumptions and on information currently available to our management. These statements relate to future events or our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements include, but are not limited to, statements about:

- our anticipated cash needs and our estimates regarding our capital requirements and our needs for additional financing;
- 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, or the FDA;
- the success, timing and cost of our current Phase 3 program for *trabodenoson* as a monotherapy and planned Phase 3 and other clinical trials and anticipated Phase 2 program for our fixed-dose combination product candidate, including statements regarding the timing of initiation and completion of the trials;
- the timing of and our ability to submit regulatory filings to the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our commercialization, marketing and manufacturing capabilities and strategy, including with respect to our potential sales force in the United States and our partnering and collaboration efforts outside the United States;
- third-party payor reimbursement for our current product candidates or any other potential products;
- our expectations regarding the clinical safety, tolerability and efficacy of our product candidates and results of our clinical trials;
- the glaucoma patient market size and the rate and degree of market adoption of our product candidates by ophthalmologists, optometrists and patients;
- the timing, cost or other aspects of a potential commercial launch of our product candidates and potential future sales of our current product candidates or any other potential products if any are approved for marketing;
- our expectations regarding licensing, acquisitions and strategic operations;
- the potential advantages of our product candidates;
- our competitors and their product candidates, including our expectations regarding those competing product candidates;
- our ability to protect and enforce our intellectual property rights, including our patented and trade secret protected proprietary rights in our product candidates; and
- anticipated trends and challenges in our business and the markets in which we operate.

These forward-looking statements are neither promises nor guarantees of future performance due to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those indicated by these forward-looking statements, including, without limitation: the possibility that we may experience slower than expected clinical site initiation or slower than expected identification and enrollment of evaluable patients; the potential for delays or problems in analyzing data or the need for additional analysis, data or patients; the potential that future pre-clinical and clinical results may not support further development of our product candidates; the potential for unexpected adverse events in the conduct of one of our clinical trials to impact our ability to continue the clinical trial or further development of a product candidate; the risk that we may encounter other unexpected hurdles or issues in the development and manufacture of our product candidates that may impact our cost, timing or progress, as well as those risks more fully discussed in the "Risk Factors" section and under "—Risks Related to Our Business" in this prospectus supplement, the section of any accompanying prospectus supplement entitled "Risk Factors" and the risk factors and cautionary statements described in other documents that we file from time to time with the SEC, specifically under "Item 1A: Risk Factors" and elsewhere in our most recent Annual Report on Form 10-K for the period ended December 31, 2015, and our Current Reports on Form 8-K.

Given these uncertainties, readers should not place undue reliance on our forward-looking statements. These forward-looking statements speak only as of the date on which the statements were made and are not guarantees of future performance. Except as may be required by applicable law, we do not undertake to update any forward-looking statements after the date of this prospectus supplement or the respective dates of documents incorporated by reference herein or therein that include forward-looking statements.

RATIO OF EARNINGS TO FIXED CHARGES

Our ratio of earnings to fixed charges for recently completed fiscal years and any required interim periods will be specified in a prospectus supplement or in a document that we file with the SEC and incorporated by reference in the future.

USE OF PROCEEDS

We intend to use the net proceeds from the sale of the securities as set forth in the applicable prospectus supplement.

DESCRIPTION OF OUR COMMON STOCK

This section describes the general terms of our common stock that we may offer from time to time. For more detailed information, a holder of our common stock should refer to our certificate of incorporation, our bylaws and our rights agreement, copies of which are filed with the SEC as exhibits to the registration statement of which this prospectus is a part.

We are authorized to issue up to 120,000,000 shares of common stock, $0.01 par value per share. As of December 31, 2015, 26,423,394 shares of common stock were outstanding.

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by the Board of Directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Stock Exchange Listing

Our common stock is listed on the NASDAQ Global Market. The trading symbol for our common stock is “ITEK.”

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Continental Stock Transfer & Trust Company. The transfer agent and registrar’s address is 17 Battery Place, New York, NY 10004.

DESCRIPTION OF OUR PREFERRED STOCK

This section describes the general terms and provisions of our preferred stock that we may offer from time to time. The applicable prospectus supplement will describe the specific terms of the shares of preferred stock offered through that prospectus supplement, which may differ from the terms we describe below. We will file a copy of the certificate of designation that contains the terms of each new series of preferred stock with the SEC each time we issue a new series of preferred stock, and these certificates of designation will be incorporated by reference into the registration statement of which this prospectus is a part. Each certificate of designation will establish the number of shares included in a designated series and fix the designation, powers, privileges, preferences and rights of the shares of each series as well as any applicable qualifications, limitations or restrictions. A holder of our preferred stock should refer to the applicable certificate of designation, our certificate of incorporation and the applicable prospectus supplement (and any related free writing prospectus that we may authorize to be provided to you) for more specific information.

Our Board of Directors currently has the authority, without further action by our stockholders, to issue up to 5,000,000 shares of preferred stock, $0.001 par value per share, in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock.

The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. No shares of preferred stock are currently outstanding.

We will incorporate by reference as an exhibit to the registration statement, which includes this prospectus, the form of any certificate of designation that describes the terms of the series of preferred stock we are offering. This description and the applicable prospectus supplement will include:

- the title and stated value;
- the number of shares authorized;
- the liquidation preference per share;
- the purchase price;
- the dividend rate, period and payment date, and method of calculation for dividends;
- whether dividends will be cumulative or non-cumulative and, if cumulative, the date from which dividends will accumulate;
- the procedures for any auction and remarketing, if any;
- the provisions for a sinking fund, if any;
- the provisions for redemption or repurchase, if applicable, and any restrictions on our ability to exercise those redemption and repurchase rights;
- any listing of the preferred stock on any securities exchange or market;
- whether the preferred stock will be convertible into our common stock, and, if applicable, the conversion price, or how it will be calculated, and the conversion period;
- whether the preferred stock will be exchangeable into debt securities, and, if applicable, the exchange price, or how it will be calculated, and the exchange period;
- voting rights, if any, of the preferred stock;

- preemptive rights, if any;
- restrictions on transfer, sale or other assignment, if any;
- a discussion of any material United States federal income tax considerations applicable to the preferred stock;
- the relative ranking and preferences of the preferred stock as to dividend rights and rights if we liquidate, dissolve or wind up our affairs;
- any limitations on issuance of any class or series of preferred stock ranking senior to or on a parity with the series of preferred stock as to dividend rights and rights if we liquidate, dissolve or wind up our affairs; and
- any other specific terms, preferences, rights or limitations of, or restrictions on, the preferred stock.

When we issue shares of preferred stock under this prospectus, the shares will fully be paid and nonassessable and will not have, or be subject to, any preemptive or similar rights.

DESCRIPTION OF OUR DEBT SECURITIES

This section describes the general terms and provisions of our debt securities that we may issue from time to time. We may issue debt securities, in one or more series, as either senior or subordinated debt or as senior or subordinated convertible debt. While the terms we have summarized below will apply generally to any future debt securities we may offer under this prospectus, the applicable prospectus supplement or free writing prospectus will describe the specific terms of any debt securities offered through that prospectus supplement or free writing prospectus. The terms of any debt securities we offer under a prospectus supplement or free writing prospectus may differ from the terms we describe below. Unless the context requires otherwise, whenever we refer to the "indentures," we also are referring to any supplemental indentures that specify the terms of a particular series of debt securities.

We will issue any senior debt securities under the senior indenture that we will enter into with the trustee named in the senior indenture. We will issue any subordinated debt securities under the subordinated indenture that we will enter into with the trustee named in the subordinated indenture. We have filed forms of these documents as exhibits to the registration statement, of which this prospectus is a part, and supplemental indentures and forms of debt securities containing the terms of the debt securities being offered will be filed as exhibits to the registration statement of which this prospectus is a part or will be incorporated by reference from reports that we file with the SEC.

The indentures will be qualified under the Trust Indenture Act of 1939, as amended, or the Trust Indenture Act. We use the term "trustee" to refer to either the trustee under the senior indenture or the trustee under the subordinated indenture, as applicable.

The following summaries of material provisions of the senior debt securities, the subordinated debt securities and the indentures are subject to, and qualified in their entirety by reference to, all of the provisions of the indenture applicable to a particular series of debt securities. We urge you to read the applicable prospectus supplement or free writing prospectus and any related free writing prospectuses related to the debt securities that we may offer under this prospectus, as well as the complete applicable indenture that contains the terms of the debt securities. Except as we may otherwise indicate, the terms of the senior indenture and the subordinated indenture are identical.

General

We will describe in the applicable prospectus supplement or free writing prospectus the terms of the series of debt securities being offered, including:

- the title;
- the principal amount being offered, and if a series, the total amount authorized and the total amount outstanding;
- any limit on the amount that may be issued;
- whether or not we will issue the series of debt securities in global form, and, if so, the terms and who the depository will be;
- the maturity date;
- whether and under what circumstances, if any, we will pay additional amounts on any debt securities held by a person who is not a United States person for tax purposes, and whether we can redeem the debt securities if we have to pay such additional amounts;
- the annual interest rate, which may be fixed or variable, or the method for determining the rate and the date interest will begin to accrue, the dates interest will be payable and the regular record dates for interest payment dates or the method for determining such dates;

- whether or not the debt securities will be secured or unsecured, and the terms of any secured debt;
- the terms of the subordination of any series of subordinated debt;
- the place where payments will be payable;
- restrictions on transfer, sale or other assignment, if any;
- our right, if any, to defer payment of interest and the maximum length of any such deferral period;
- the date, if any, after which, the conditions upon which, and the price at which, we may, at our option, redeem the series of debt securities pursuant to any optional or provisional redemption provisions and the terms of those redemption provisions;
- the date, if any, on which, and the price at which we are obligated, pursuant to any mandatory sinking fund or analogous fund provisions or otherwise, to redeem, or at the holder's option, to purchase, the series of debt securities and the currency or currency unit in which the debt securities are payable;
- whether the indenture will restrict our ability or the ability of our subsidiaries to:
  - incur additional indebtedness;
  - issue additional securities;
  - create liens;
  - pay dividends or make distributions in respect of our capital stock or the capital stock of our subsidiaries;
  - redeem capital stock;
  - place restrictions on our subsidiaries' ability to pay dividends, make distributions or transfer assets;
  - make investments or other restricted payments;
  - sell or otherwise dispose of assets;
  - enter into sale-leaseback transactions;
  - engage in transactions with stockholders or affiliates;
  - issue or sell stock of our subsidiaries; or
  - effect a consolidation or merger;
- whether the indenture will require us to maintain any interest coverage, fixed charge, cash flow-based, asset-based or other financial ratios;
- a discussion of certain material or special United States federal income tax considerations applicable to the debt securities;
- information describing any book-entry features;
- provisions for a sinking fund purchase or other analogous fund, if any;
- the applicability of the provisions in the indenture on discharge;
- whether the debt securities are to be offered at a price such that they will be deemed to be offered at an "original issue discount" as defined in paragraph (a) of Section 1273 of the Internal Revenue Code of 1986, as amended;
- the denominations in which we will issue the series of debt securities, if other than denominations of $1,000 and any integral multiple thereof;

- the currency of payment of debt securities if other than U.S. dollars and the manner of determining the equivalent amount in U.S. dollars; and
- any other specific terms, preferences, rights or limitations of, or restrictions on, the debt securities, including any additional events of default or covenants provided with respect to the debt securities, and any terms that may be required by us or advisable under applicable laws or regulations or advisable in connection with the marketing of the debt securities.

Conversion or Exchange Rights

We will set forth in the applicable prospectus supplement or free writing prospectus the terms on which a series of debt securities may be convertible into or exchangeable for our common stock, our preferred stock or other securities (including securities of a third-party). We will include provisions as to whether conversion or exchange is mandatory, at the option of the holder or at our option. We may include provisions pursuant to which the number of shares of our common stock, our preferred stock or other securities (including securities of a third-party) that the holders of the series of debt securities receive would be subject to adjustment.

Consolidation, Merger or Sale

Unless we provide otherwise in the prospectus supplement or free writing prospectus applicable to a particular series of debt securities, the indentures will not contain any covenant that restricts our ability to merge or consolidate, or sell, convey, transfer or otherwise dispose of all or substantially all of our assets. However, any successor to or acquirer of such assets must assume all of our obligations under the indentures or the debt securities, as appropriate. If the debt securities are convertible into or exchangeable for other securities of ours or securities of other entities, the person with whom we consolidate or merge or to whom we sell all of our property must make provisions for the conversion of the debt securities into securities that the holders of the debt securities would have received if they had converted the debt securities before the consolidation, merger or sale.

Events of Default Under the Indenture

Unless we provide otherwise in the prospectus supplement or free writing prospectus applicable to a particular series of debt securities, the following are events of default under the indentures with respect to any series of debt securities that we may issue:

- if we fail to pay interest when due and payable and our failure continues for 90 days and the time for payment has not been extended;
- if we fail to pay the principal, premium or sinking fund payment, if any, when due and payable at maturity, upon redemption or repurchase or otherwise, and the time for payment has not been extended;
- if we fail to observe or perform any other covenant contained in the debt securities or the indentures, other than a covenant specifically relating to another series of debt securities, and our failure continues for 90 days after we receive notice from the trustee or holders of at least 25% in aggregate principal amount of the outstanding debt securities of the applicable series; and
- if specified events of bankruptcy, insolvency or reorganization occur.

We will describe in each applicable prospectus supplement or free writing prospectus any additional events of default relating to the relevant series of debt securities.

If an event of default with respect to debt securities of any series occurs and is continuing, other than an event of default specified in the last bullet point above, the trustee or the holders of at least 25% in aggregate principal amount of the outstanding debt securities of that series, by notice to us in writing, and to the trustee if notice is given by such holders, may declare the unpaid principal, premium, if any, and accrued interest, if any,

due and payable immediately. If an event of default specified in the last bullet point above occurs with respect to us, the unpaid principal, premium, if any, and accrued interest, if any, of each issue of debt securities then outstanding shall be due and payable without any notice or other action on the part of the trustee or any holder.

The holders of a majority in principal amount of the outstanding debt securities of an affected series may waive any default or event of default with respect to the series and its consequences, except defaults or events of default regarding payment of principal, premium, if any, or interest, unless we have cured the default or event of default in accordance with the indenture. Any waiver shall cure the default or event of default.

Subject to the terms of the indentures, if an event of default under an indenture shall occur and be continuing, the trustee will be under no obligation to exercise any of its rights or powers under such indenture at the request or direction of any of the holders of the applicable series of debt securities, unless such holders have offered the trustee reasonable indemnity or security satisfactory to it against any loss, liability or expense. The holders of a majority in principal amount of the outstanding debt securities of any series will have the right to direct the time, method and place of conducting any proceeding for any remedy available to the trustee, or exercising any trust or power conferred on the trustee, with respect to the debt securities of that series, provided that:

- the direction so given by the holder is not in conflict with any law or the applicable indenture; and
- subject to its duties under the Trust Indenture Act, the trustee need not take any action that might involve it in personal liability or might be unduly prejudicial to the holders not involved in the proceeding.

A holder of the debt securities of any series will have the right to institute a proceeding under the indentures or to appoint a receiver or trustee, or to seek other remedies if:

- the holder has given written notice to the trustee of a continuing event of default with respect to that series;
- the holders of at least 25% in aggregate principal amount of the outstanding debt securities of that series have made written request, and such holders have offered reasonable indemnity to the trustee or security satisfactory to it against any loss, liability or expense or to be incurred in compliance with instituting the proceeding as trustee; and
- the trustee does not institute the proceeding, and does not receive from the holders of a majority in aggregate principal amount of the outstanding debt securities of that series other conflicting directions within 90 days after the notice, request and offer.

These limitations do not apply to a suit instituted by a holder of debt securities if we default in the payment of the principal, premium, if any, or interest on, the debt securities, or other defaults that may be specified in the applicable prospectus supplement or free writing prospectus.

We will periodically file statements with the trustee regarding our compliance with specified covenants in the indentures.

Modification of Indenture; Waiver

Subject to the terms of the indenture for any series of debt securities that we may issue, we and the trustee may change an indenture without the consent of any holders with respect to the following specific matters:

- to fix any ambiguity, defect or inconsistency in the indenture;
- to comply with the provisions described above under “Description of Our Debt Securities—Consolidation, Merger or Sale;”
- to comply with any requirements of the SEC in connection with the qualification of any indenture under the Trust Indenture Act;

- to add to, delete from or revise the conditions, limitations, and restrictions on the authorized amount, terms, or purposes of issue, authentication and delivery of debt securities, as set forth in the indenture;
- to provide for the issuance of and establish the form and terms and conditions of the debt securities of any series as provided under "Description of Our Debt Securities—General," to establish the form of any certifications required to be furnished pursuant to the terms of the indenture or any series of debt securities, or to add to the rights of the holders of any series of debt securities;
- to evidence and provide for the acceptance of appointment hereunder by a successor trustee;
- to provide for uncertificated debt securities and to make all appropriate changes for such purpose;
- to add to our covenants such new covenants, restrictions, conditions or provisions for the benefit of the holders, to make the occurrence, or the occurrence and the continuance, of a default in any such additional covenants, restrictions, conditions or provisions an event of default or to surrender any right or power conferred to us in the indenture; or
- to change anything that does not materially adversely affect the interests of any holder of debt securities of any series.

In addition, under the indentures, the rights of holders of a series of debt securities may be changed by us and the trustee with the written consent of the holders of at least a majority in aggregate principal amount of the outstanding debt securities of each series that is affected. However, subject to the terms of the indenture for any series of debt securities that we may issue or as otherwise provided in the prospectus supplement or free writing prospectus applicable to a particular series of debt securities, we and the trustee may make the following changes only with the consent of each holder of any outstanding debt securities affected:

- extending the stated maturity of the series of debt securities;
- reducing the principal amount, reducing the rate of or extending the time of payment of interest, or reducing any premium payable upon the redemption or repurchase of any debt securities; or
- reducing the percentage of debt securities, the holders of which are required to consent to any amendment, supplement, modification or waiver.

Discharge

Each indenture provides that, subject to the terms of the indenture and any limitation otherwise provided in the prospectus supplement or free writing prospectus applicable to a particular series of debt securities, we can elect to be discharged from our obligations with respect to one or more series of debt securities, except for specified obligations, including obligations to:

- register the transfer or exchange of debt securities of the series;
- replace stolen, lost or mutilated debt securities of the series;
- maintain paying agencies;
- hold monies for payment in trust;
- recover excess money held by the trustee;
- compensate and indemnify the trustee; and
- appoint any successor trustee.

In order to exercise our rights to be discharged, we must deposit with the trustee money or government obligations sufficient to pay all the principal of, any premium and interest on, the debt securities of the series on the dates payments are due.

Form, Exchange and Transfer

We will issue the debt securities of each series only in fully registered form without coupons and, unless we otherwise specify in the applicable prospectus supplement or free writing prospectus, in denominations of $1,000 and any integral multiple thereof. The indentures provide that we may issue debt securities of a series in temporary or permanent global form and as book-entry securities that will be deposited with, or on behalf of, The Depository Trust Company or another depository named by us and identified in a prospectus supplement or free writing prospectus with respect to that series.

At the option of the holder, subject to the terms of the indentures and the limitations applicable to global securities described in the applicable prospectus supplement or free writing prospectus, the holder of the debt securities of any series can exchange the debt securities for other debt securities of the same series, in any authorized denomination and of like tenor and aggregate principal amount.

Subject to the terms of the indentures and the limitations applicable to global securities set forth in the applicable prospectus supplement or free writing prospectus, holders of the debt securities may present the debt securities for exchange or for registration of transfer, duly endorsed or with the form of transfer endorsed thereon duly executed if so required by us or the security registrar, at the office of the security registrar or at the office of any transfer agent designated by us for this purpose. Unless otherwise provided in the debt securities that the holder presents for transfer or exchange, we will make no service charge for any registration of transfer or exchange, but we may require payment of any taxes or other governmental charges.

We will name in the applicable prospectus supplement or free writing prospectus the security registrar, and any transfer agent in addition to the security registrar, that we initially designate for any debt securities. We may at any time designate additional transfer agents or rescind the designation of any transfer agent or approve a change in the office through which any transfer agent acts, except that we will be required to maintain a transfer agent in each place of payment for the debt securities of each series. If we elect to redeem the debt securities of any series, we will not be required to:

- issue, register the transfer of, or exchange any debt securities of that series during a period beginning at the opening of business 15 days before the day of mailing of a notice of redemption of any debt securities that may be selected for redemption and ending at the close of business on the day of the mailing; or
- register the transfer of or exchange any debt securities so selected for redemption, in whole or in part, except the unredeemed portion of any debt securities we are redeeming in part.

Information Concerning the Trustee

The trustee, other than during the occurrence and continuance of an event of default under an indenture, undertakes to perform only those duties as are specifically set forth in the applicable indenture. Upon an event of default under an indenture, the trustee must use the same degree of care as a prudent person would exercise or use in the conduct of his or her own affairs.

Subject to this provision, the trustee is under no obligation to exercise any of the powers given it by the indentures at the request of any holder of debt securities unless it is offered reasonable security and indemnity against the costs, expenses and liabilities that it might incur.

Payment and Paying Agents

Unless we otherwise indicate in the applicable prospectus supplement or free writing prospectus, we will make payment of the interest on any debt securities on any interest payment date to the person in whose name the debt securities, or one or more predecessor securities, are registered at the close of business on the regular record date for the interest.

We will pay principal of and any premium and interest on the debt securities of a particular series at the office of the paying agents designated by us, except that unless we otherwise indicate in the applicable prospectus supplement or free writing prospectus, we will make interest payments by check that we will mail to the holder or by wire transfer to certain holders. Unless we otherwise indicate in the applicable prospectus supplement or free writing prospectus, we will designate the corporate trust office of the trustee as our sole paying agent for payments with respect to debt securities of each series. We will name in the applicable prospectus supplement or free writing prospectus any other paying agents that we initially designate for the debt securities of a particular series. We will maintain a paying agent in each place of payment for the debt securities of a particular series.

All money we pay to a paying agent or the trustee for the payment of the principal of or any premium or interest on any debt securities that remains unclaimed at the end of two years after such principal, premium or interest has become due and payable will be repaid to us, and the holder of the debt security thereafter may look only to us for payment thereof.

Governing Law

The indentures and the debt securities will be governed by and construed in accordance with the laws of the State of New York, except to the extent that the Trust Indenture Act is applicable.

Ranking of Debt Securities

The subordinated debt securities will be subordinate and junior in priority of payment to certain of our other indebtedness to the extent described in a prospectus supplement or free writing prospectus. The subordinated indenture does not limit the amount of subordinated debt securities that we may issue. It also does not limit us from issuing any other secured or unsecured debt.

The senior debt securities will rank equally in right of payment to all our other senior unsecured debt. The senior indenture does not limit the amount of senior debt securities that we may issue. It also does not limit us from issuing any other secured or unsecured debt.

DESCRIPTION OF OUR WARRANTS

The following description, together with the additional information we include in any applicable prospectus supplements or free writing prospectus, summarizes the material terms and provisions of the warrants that we may offer under this prospectus, which may consist of warrants to purchase common stock, preferred stock and/or debt securities in one or more series. Warrants may be offered independently or together with common stock, preferred stock and/or debt securities offered by any prospectus supplement or free writing prospectus, and may be attached to or separate from those securities. While the terms we have summarized below will generally apply to any future warrants we may offer under this prospectus, we will describe the particular terms of any warrants that we may offer in more detail in the applicable prospectus supplement or free writing prospectus. The terms of any warrants we offer under a prospectus supplement or free writing prospectus may differ from the terms we describe below.

We will issue the warrants under a warrant agreement which we will enter into with a warrant agent to be selected by us. Forms of these warrant agreements and forms of the warrant certificates representing the warrants, and the complete warrant agreements and forms of warrant certificates containing the terms of the warrants being offered, will be filed as exhibits to the registration statement of which this prospectus is a part or will be incorporated by reference from reports that we file with the SEC. We use the term "warrant agreement" to refer to any of these warrant agreements. We use the term "warrant agent" to refer to the warrant agent under any of these warrant agreements. The warrant agent will act solely as an agent of ours in connection with the warrants and will not act as an agent for the holders or beneficial owners of the warrants.

The following summaries of material provisions of the warrants and the warrant agreements are subject to, and qualified in their entirety by reference to, all the provisions of the warrant agreement applicable to a particular series of warrants. We urge you to read the applicable prospectus supplements or free writing prospectus related to the warrants that we sell under this prospectus, as well as the complete warrant agreements that contain the terms of the warrants.

General

We will describe in the applicable prospectus supplement or free writing prospectus the terms relating to a series of warrants. If warrants for the purchase of debt securities are offered, the prospectus supplement or free writing prospectus will describe the following terms, to the extent applicable:

- the offering price and the aggregate number of warrants offered;
- the currencies in which the warrants are being offered;
- the designation, aggregate principal amount, currencies, denominations and terms of the series of debt securities that can be purchased if a holder exercises a warrant;
- the designation and terms of any series of debt securities with which the warrants are being offered and the number of warrants offered with each such debt security;
- the date on and after which the holder of the warrants can transfer them separately from the related series of debt securities;
- the principal amount of the series of debt securities that can be purchased if a holder exercises a warrant and the price at which and currencies in which such principal amount may be purchased upon exercise;
- the terms of any rights to redeem or call the warrants;
- the date on which the right to exercise the warrants begins and the date on which such right expires;
- federal income tax consequences of holding or exercising the warrants; and
- any other specific terms, preferences, rights or limitations of, or restrictions on, the warrants.

Warrants for the purchase of debt securities will be in registered form only.

If warrants for the purchase of common stock or preferred stock are offered, the prospectus supplement or free writing prospectus will describe the following terms, to the extent applicable:

- the offering price and the aggregate number of warrants offered;
- the total number of shares that can be purchased if a holder of the warrants exercises them and, in the case of warrants for preferred stock, the designation, total number and terms of the series of preferred stock that can be purchased upon exercise;
- the designation and terms of any series of preferred stock with which the warrants are being offered and the number of warrants being offered with each share of common stock or preferred stock;
- the date on and after which the holder of the warrants can transfer them separately from the related common stock or series of preferred stock;
- the number of shares of common stock or preferred stock that can be purchased if a holder exercises the warrant and the price at which such common stock or preferred stock may be purchased upon exercise, including, if applicable, any provisions for changes to or adjustments in the exercise price and in the securities or other property receivable upon exercise;
- the terms of any rights to redeem or call, or accelerate the expiration of, the warrants;
- the date on which the right to exercise the warrants begins and the date on which that right expires;
- federal income tax consequences of holding or exercising the warrants; and
- any other specific terms, preferences, rights or limitations of, or restrictions on, the warrants.

Warrants for the purchase of common stock or preferred stock will be in registered form only.

Exercise of Warrants

Each holder of a warrant is entitled to purchase the principal amount of debt securities or number of shares of common stock or preferred stock, as the case may be, at the exercise price described in the applicable prospectus supplement or free writing prospectus. After the close of business on the day when the right to exercise terminates (or a later date if we extend the time for exercise), unexercised warrants will become void.

A holder of warrants may exercise them by following the general procedure outlined below:

- delivering to the warrant agent the payment required by the applicable prospectus supplement or free writing prospectus to purchase the underlying security;
- properly completing and signing the reverse side of the warrant certificate representing the warrants; and
- delivering the warrant certificate representing the warrants to the warrant agent within five business days of the warrant agent receiving payment of the exercise price.

If you comply with the procedures described above, your warrants will be considered to have been exercised when the warrant agent receives payment of the exercise price, subject to the transfer books for the securities issuable upon exercise of the warrant not being closed on such date. After you have completed those procedures and subject to the foregoing, we will, as soon as practicable, issue and deliver to you the debt securities, common stock or preferred stock that you purchased upon exercise. If you exercise fewer than all of the warrants represented by a warrant certificate, a new warrant certificate will be issued to you for the unexercised amount of warrants. Holders of warrants will be required to pay any tax or governmental charge that may be imposed in connection with transferring the underlying securities in connection with the exercise of the warrants.

Amendments and Supplements to the Warrant Agreements

We may amend or supplement a warrant agreement without the consent of the holders of the applicable warrants to cure ambiguities in the warrant agreement, to cure or correct a defective provision in the warrant

agreement, or to provide for other matters under the warrant agreement that we and the warrant agent deem necessary or desirable, so long as, in each case, such amendments or supplements do not materially adversely affect the interests of the holders of the warrants.

Warrant Adjustments

Unless the applicable prospectus supplement or free writing prospectus states otherwise, the exercise price of, and the number of securities covered by, a common stock warrant or preferred stock warrant will be adjusted proportionately if we subdivide or combine our common stock or preferred stock, as applicable. In addition, unless the prospectus supplement or free writing prospectus states otherwise, if we, without receiving payment:

- issue capital stock or other securities convertible into or exchangeable for common stock or preferred stock, or any rights to subscribe for, purchase or otherwise acquire any of the foregoing, as a dividend or distribution to holders of our common stock or preferred stock;
- pay any cash to holders of our common stock or preferred stock other than a cash dividend paid out of our current or retained earnings or other than in accordance with the terms of the preferred stock;
- issue any evidence of our indebtedness or rights to subscribe for or purchase our indebtedness to holders of our common stock or preferred stock; or
- issue common stock or preferred stock or additional stock or other securities or property to holders of our common stock or preferred stock by way of spinoff, split-up, reclassification, combination of shares or similar corporate rearrangement,

then the holders of common stock warrants and preferred stock warrants, as applicable, will be entitled to receive upon exercise of the warrants, in addition to the securities otherwise receivable upon exercise of the warrants and without paying any additional consideration, the amount of stock and other securities and property such holders would have been entitled to receive had they held the common stock or preferred stock, as applicable, issuable under the warrants on the dates on which holders of those securities received or became entitled to receive such additional stock and other securities and property.

Except as stated above or as otherwise set forth in the applicable prospectus supplement or free writing prospectus, the exercise price and number of securities covered by a common stock warrant and preferred stock warrant, and the amounts of other securities or property to be received, if any, upon exercise of those warrants, will not be adjusted or provided for if we issue those securities or any securities convertible into or exchangeable for those securities, or securities carrying the right to purchase those securities or securities convertible into or exchangeable for those securities.

Holders of common stock warrants and preferred stock warrants may have additional rights under the following circumstances:

- certain reclassifications, capital reorganizations or changes of the common stock or preferred stock, as applicable;
- certain share exchanges, mergers, or similar transactions involving us and which result in changes of the common stock or preferred stock, as applicable; or
- certain sales or dispositions to another entity of all or substantially all of our property and assets.

If one of the above transactions occurs and holders of our common stock or preferred stock are entitled to receive stock, securities or other property with respect to or in exchange for their securities, the holders of the common stock warrants and preferred stock warrants then outstanding, as applicable, will be entitled to receive upon exercise of their warrants the kind and amount of shares of stock and other securities or property that they would have received upon the applicable transaction if they had exercised their warrants immediately before the transaction.

DESCRIPTION OF OUR UNITS

This section outlines some of the provisions of the units and the unit agreements. This information may not be complete in all respects and is qualified entirely by reference to the unit agreement with respect to the units of any particular series. The specific terms of any series of units will be described in the applicable prospectus supplement or free writing prospectus. If so described in a particular prospectus supplement or free writing prospectus, the specific terms of any series of units may differ from the general description of terms presented below.

As specified in the applicable prospectus supplement, we may issue units consisting of one or more shares of common stock, shares of preferred stock, debt securities, warrants or any combination of such securities.

The applicable prospectus supplement will specify the following terms of any units in respect of which this prospectus is being delivered:

- the terms of the units and of any of the shares of common stock, shares of preferred stock, debt securities, or warrants comprising the units, including whether and under what circumstances the securities comprising the units may be traded separately;
- a description of the terms of any unit agreement governing the units;
- if appropriate, a discussion of material U.S. federal income tax considerations; and
- a description of the provisions for the payment, settlement, transfer or exchange of the units.

DESCRIPTION OF CERTAIN PROVISIONS OF DELAWARE LAW AND OUR CERTIFICATE OF INCORPORATION AND BYLAWS

We are organized as a Delaware corporation. The following is a summary of our certificate of incorporation and bylaws and certain provisions of the Delaware General Corporation Law, or the DGCL. Because it is a summary, it does not contain all the information that may be important to you. If you want more information, you should read our entire certificate of incorporation and bylaws, copies of which are filed with the SEC as exhibits to the registration statement of which this prospectus is a part. See "Where You Can Find Additional Information," or refer to the provisions of Delaware law.

Classification of Directors

Our certificate of incorporation provides for the division of our Board of Directors into three classes serving staggered three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote of the holders of 75% of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our Board of Directors, however occurring, including a vacancy resulting from an increase in the size of our Board of Directors, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our Board of Directors.

Special Meetings

Our certificate of incorporation and bylaws provide that only a majority of the members of our Board of Directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Indemnification

Our certificate of incorporation and our bylaws, as amended, provide that we shall indemnify our directors and officers to the fullest extent permitted by law. In addition, we have previously entered into and intend to enter into new agreements to indemnify our directors and executive officers. These agreements will, among other things, indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of us or that person's status as a member of our Board of Directors.

Section 203 of the Delaware General Corporation Law

We are subject to the provisions of Section 203 of the DGCL. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our Board of Directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the

voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or

- at or after the time the stockholder became interested, the business combination was approved by our Board of Directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

PLAN OF DISTRIBUTION

We may sell our securities from time to time in one or more transactions. We may sell our securities to or through agents, underwriters, dealers, remarketing firms or other third parties or directly to one or more purchasers or through a combination of any of these methods. In some cases, we or dealers acting with us or on behalf of us may also purchase our securities and reoffer them to the public. We may also offer and sell, or agree to deliver, our securities pursuant to, or in connection with, any option agreement or other contractual arrangement.

Agents whom we designate may solicit offers to purchase our securities.

- We will name any agent involved in offering or selling our securities, and disclose any commissions that we will pay to the agent, in the applicable prospectus supplement.
- Unless we indicate otherwise in the applicable prospectus supplement, agents will act on a best efforts basis for the period of their appointment.
- Agents may be deemed to be underwriters under the Securities Act, of any of our securities that they offer or sell.

We may use an underwriter or underwriters in the offer or sale of our securities.

- If we use an underwriter or underwriters, we will execute an underwriting agreement with the underwriter or underwriters at the time that we reach an agreement for the sale of our securities.
- We will include the names of the specific managing underwriter or underwriters, as well as the names of any other underwriters, and the terms of the transactions, including the compensation the underwriters and dealers will receive, in the applicable prospectus supplement.
- The underwriters will use the applicable prospectus supplement, together with the prospectus, to sell our securities.

We may use a dealer to sell our securities.

- If we use a dealer, we will sell our securities to the dealer, as principal.
- The dealer will then sell our securities to the public at varying prices that the dealer will determine at the time it sells our securities.
- We will include the name of the dealer and the terms of the transactions with the dealer in the applicable prospectus supplement.

We may solicit directly offers to purchase our securities, and we may directly sell our securities to institutional or other investors. We will describe the terms of direct sales in the applicable prospectus supplement.

We may engage in at-the-market offerings into an existing trading market in accordance with Rule 415(a)(4) of the Securities Act.

We may indemnify agents, underwriters and dealers against certain liabilities, including liabilities under the Securities Act. Agents, underwriters and dealers, or their affiliates, may be customers of, engage in transactions with or perform services for us or our respective affiliates, in the ordinary course of business.

We may authorize agents and underwriters to solicit offers by certain institutions to purchase our securities at the public offering price under delayed delivery contracts.

- If we use delayed delivery contracts, we will disclose that we are using them in the prospectus supplement and will tell you when we will demand payment and when delivery of our securities will be made under the delayed delivery contracts.

- These delayed delivery contracts will be subject only to the conditions that we describe in the prospectus supplement.
- We will describe in the applicable prospectus supplement the commission that underwriters and agents soliciting purchases of our securities under delayed delivery contracts will be entitled to receive.

Unless otherwise specified in connection with a particular underwritten offering of our securities, the underwriters will not be obligated to purchase offered securities unless specified conditions are satisfied, and if the underwriters do purchase any offered securities, they will purchase all offered securities.

In connection with underwritten offerings of the offered securities and in accordance with applicable law and industry practice, the underwriters in certain circumstances are permitted to engage in certain transactions that stabilize the price of our securities. Such transactions consist of bids or purchases for the purpose of pegging, fixing or maintaining the price of our securities. If the underwriters create a short position in our securities in connection with the offering (i.e., if they sell more securities than are set forth on the cover page of the applicable prospectus supplement), the underwriters may reduce that short position by purchasing our securities in the open market or as otherwise provided in the applicable prospectus supplement. The underwriters may also impose a penalty bid, whereby selling concessions allowed to dealers participating in the offering may be reclaimed if the securities sold by them are repurchased in connection with stabilization transactions. In general, purchases of a security for the purpose of stabilization or to reduce a short position could cause the price of the security to be higher than it might be in the absence of such purchases. The imposition of a penalty bid might also have an effect on the price of our securities to the extent that it were to discourage resales of our securities. The underwriters are not required to engage in these activities and may end any of these activities at any time.

We may effect sales of securities in connection with forward sale, option or other types of agreements with third parties. Any distribution of securities pursuant to any forward sale agreement may be effected from time to time in one or more transactions that may take place through a stock exchange, including block trades or ordinary broker’s transactions, or through broker-dealers acting either as principal or agent, or through privately-negotiated transactions, or through an underwritten public offering, or through a combination of any such methods of sale, at market prices prevailing at the time of sale, prices relating to such prevailing market prices or at negotiated or fixed prices.

The specific terms of the lock-up provisions, if any, in respect of any given offering will be described in the applicable prospectus supplement.

LEGAL MATTERS

The validity of the securities being offered by this prospectus will be passed upon by Goodwin Procter LLP, Boston, Massachusetts. Additional legal matters may be passed upon for us or any underwriters, dealers or agents, by counsel that we will name in the applicable prospectus supplement.

EXPERTS

The consolidated financial statements of Inotek Pharmaceuticals Corporation as of December 31, 2015 and 2014 and for the years then ended, incorporated in this Prospectus by reference from the Inotek Pharmaceuticals Corporation Annual Report on Form 10-K for the year ended December 31, 2015, have been audited by RSM US LLP, an independent registered public accounting firm, as stated in their reports incorporated herein by reference, and have been incorporated in reliance upon such reports and upon the authority of such firm as experts in accounting.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

This prospectus is part of a registration statement that we have filed with the SEC. Certain information in the registration statement has been omitted from this prospectus in accordance with the rules of the SEC. We are subject to the information requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and, in accordance therewith, file annual, quarterly and special reports, proxy statements and other information with the SEC. You may read and copy any document we file at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C., 20549. You may call the SEC at 1-800-SEC-0330 for further information on the operation of the Public Reference Room. These documents also may be accessed through the SEC's Electronic Data Gathering, Analysis and Retrieval system, or EDGAR, via electronic means, including the SEC's home page on the Internet (*www.sec.gov*).

We have the authority to designate and issue more than one class or series of stock having various preferences, conversion and other rights, voting powers, restrictions, limitations as to dividends, qualifications and terms and conditions of redemption. We will furnish a full statement of the relative rights and preferences of each class or series of our stock which has been so designated and any restrictions on the ownership or transfer of our stock to any stockholder upon request and without charge. Written requests for such copies should be directed to Inotek Pharmaceuticals Corporation, 91 Hartwell Avenue, Lexington, Massachusetts, 02421, Attention: Secretary, or by telephone request to (781) 676-2100. Our website is located at *http://www.inotekpharma.com*. Information contained on our website is not incorporated by reference into this prospectus, and you should not consider any information on, or that can be accessed from, our website as part of this prospectus or any accompanying prospectus supplement.

INCORPORATION OF CERTAIN INFORMATION BY REFERENCE

The SEC allows us to incorporate by reference the information and reports we file with it, which means that we can disclose important information to you by referring you to these documents. The information incorporated by reference is an important part of this prospectus, and information that we file after the date hereof with the SEC will automatically update and supersede the information already incorporated by reference. We are incorporating by reference the documents listed below, which we have already filed with the SEC, and any future filings we make with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act, except as to any portion of any future report or document that is not deemed filed under such provisions, after the date of this prospectus and prior to the termination of this offering:

- Annual Report on Form 10-K for the year ended December 31, 2015, as filed with the SEC on March 23, 2016;
- Current Reports on Form 8-K filed with the SEC on January 12, 2016, February 26, 2016, March 11, 2016, March 25, 2016 and April 4, 2016 (in each case, except for information contained therein which is furnished rather than filed); and
- The description of our common stock contained in our registration statement on Form 8-A, which was filed with the SEC on February 2, 2015, including any amendment or report filed for the purpose of updating such description.

Upon request, we will provide, without charge, to each person, including any beneficial owner, to whom a copy of this prospectus is delivered a copy of the documents incorporated by reference into this prospectus. You may request a copy of these filings, and any exhibits we have specifically incorporated by reference as an exhibit in this prospectus, at no cost by writing or telephoning us at the following:

Inotek Pharmaceuticals Corporation, 91 Hartwell Avenue, Lexington, Massachusetts, 02421, Attention: Secretary, (781) 676-2100.

You may also access these documents, free of charge on the SEC's website at *www.sec.gov* or on our website at *www.inotekpharma.com*. Information contained on our website is not incorporated by reference into this prospectus, and you should not consider any information on, or that can be accessed from, our website as part of this prospectus or any accompanying prospectus supplement.

This prospectus is part of a registration statement we filed with the SEC. We have incorporated exhibits into this registration statement. You should read the exhibits carefully for provisions that may be important to you.

We have not authorized anyone to provide you with information other than what is incorporated by reference or provided in this prospectus or any prospectus supplement. We are not making an offer of these securities in any state where such offer is not permitted. You should not assume that the information in this prospectus or in the documents incorporated by reference is accurate as of any date other than the date on the front of this prospectus or those documents.

Shares

Rocket Pharmaceuticals, Inc.

Common Stock

PROSPECTUS SUPPLEMENT

Joint Bookrunning Managers

Cowen **Evercore ISI**