



Rocket Pharmaceuticals and Inotek Pharmaceuticals Complete Merger Transaction

January 4, 2018

- *Strong Support by Shareholders Approving Merger Proposal* -

- *Multi-Platform Approach to Develop Gene Therapies for Rare Diseases* -

- *Multiple Programs in the Clinic in 2018; Data from One or More Programs During the Year* -

NEW YORK--([BUSINESS WIRE](#))--[Rocket Pharmaceuticals, Ltd.](#), a leading U.S.-based multi-platform gene therapy company addressing challenging rare diseases, today announced the completion of its merger with Inotek Pharmaceuticals Corporation (“Inotek”). The combined company (“Company”) will be named Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”) and will focus on advancing a pipeline of gene therapy programs targeting rare and undertreated diseases. Rocket’s common stock will be listed on the NASDAQ Global Market under the symbol “RCKT” and is expected to be begin trading on January 5, 2018. Rocket is based in New York City and led by President and Chief Executive Officer Gaurav Shah, M.D., who previously was a Global Program Head in the Cell & Gene Therapies Unit at Novartis.

“The support for this transaction by both Inotek and Rocket shareholders was evident today, underscoring support for our long-term growth strategy to become a fully-integrated, multi-platform gene therapy company,” said Dr. Shah. “The closing of this merger provides immediate value to grow our operations, execute on our clinical development goals, and expand our in-house manufacturing and analytics capabilities. As we enter this next stage of growth as a publicly traded company, we are focused on driving Company value by bringing our current pipeline of five programs to major value inflection points as rapidly as possible, and achieving first mover advantage in these markets.”

Rocket’s Pipeline

Rocket utilizes a multi-platform development approach that leverages the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene delivery methods and is initially targeting devastating rare diseases in children that lead to early mortality in the absence of bone marrow transplant or other invasive procedures.

The Company’s lead program, a Phase 1/2 LVV-based gene therapy for Fanconi Anemia (FA), is currently in clinical trials with academic partners in the U.S. and Europe. FA causes genetic instability due to mutations in DNA repair genes resulting in early bone marrow failure and malignancy. Early results in FA patients have demonstrated clinical engraftment of *ex vivo*-transduced autologous hematopoietic stem cells (HSCs). The proportion of gene-corrected cells increases over time, confirming the selective advantage of gene-corrected cells in the bone marrow without requiring conditioning (i.e. destruction of bone marrow prior to transplant). Functional correction and clinical proof of concept have also been observed. Both blood and marrow cells demonstrate resistance to DNA-damaging agents (sensitivity to DNA-damaging agents is a diagnostic feature of FA). Patients demonstrated stable or improving blood cell counts during the months following treatment despite decreases noted during the months and years preceding gene therapy. Additional patient data are expected in 2018, with a registration study anticipated to start in 2019.

Three additional LVV-based programs are currently in preclinical development and target Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO). The LAD-I program is expected to advance into the clinic in 2018, with the PKD and IMO programs to follow in 2019.

An undisclosed AAV-based gene therapy program is expected to enter the clinic in the next year and has demonstrated encouraging histological correction of the disease phenotype. This program targets a monogenic pediatric disease with early mortality and represents the first gene therapy being developed for this large class of indications.

Rocket’s Management

The combined Company’s executive management team will be led by Dr. Shah and will consist of: Jonathan Schwartz, M.D., Chief Medical Officer, who led several biologics approvals as Vice President of Clinical Development at ImClone Systems/Eli Lilly and Company; Kinnari Patel, Pharm.D., MBA, newly appointed Chief Operating Officer, who led regulatory filings for six rare disease agents as well as for Opdivo while at Bristol-Myers Squibb; and Brian Batchelder, MBA, Vice President of Finance, who previously served as Chief Financial Officer of ImClone Systems, a subsidiary of Eli Lilly and Company.

In addition, Rocket appointed Claudine Prowse, Ph.D., as Head of Corporate Development and Investor Relations Officer. Previously, she was Head

of Strategy at Inotek, where she was integral to the merger transaction with Rocket Pharmaceuticals, Ltd. Prior to that, she was Vice President of Investor Relations at Biogen.

About the Merger

Prior to the closing of the merger, Inotek effected a 1 for 4 reverse split of its common stock. Following the reverse stock split and closing of the merger, there will be approximately 33.1 million shares of the combined company's common stock outstanding with prior Rocket shareholders owning approximately 79.4% and prior Inotek shareholders owning approximately 20.6%. Based on the reverse stock split, the conversion rate of the combined Company's \$52.0 million of 5.75% Convertible Senior Notes due 2021 will automatically be adjusted from 124.7505 shares of common stock per \$1,000 principal amount of the notes to 31.1876 shares of common stock per \$1,000 principal amount of the notes. Cash, cash equivalents and short-term investments for the combined Company at closing were approximately \$117.2 million.

About Rocket Pharmaceuticals, Inc.

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

Forward-Looking Statements

Various statements in this release concerning the Company's future expectations, plans and prospects, including without limitation, the Company's expectations regarding the safety, effectiveness and timing of products that the Company may develop, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), and the safety, effectiveness and timing of related pre-clinical studies and clinical studies, may constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe", "expect", "anticipate", "intend", "plan", "will give", "estimate", "seek", "will", "may", "suggest" or similar terms, variations of such terms or the negative of those terms. Although the Company believes that the expectations reflected in the forward-looking statements are reasonable, the Company cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, the Company's ability to successfully demonstrate the efficacy and safety of such products and pre-clinical and clinical studies, its gene therapy programs, the pre-clinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of the Company's product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, the Company's and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of the Company's product candidates, the Company's ability to manage operating expenses, the Company's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, the Company's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in the Definitive Proxy Statement on Schedule 14A filed by the Company with the Securities and Exchange Commission in connection with the merger. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and the Company undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Contacts

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