Rocket Pharmaceuticals Announces Clearance of IND for RP-A501 Gene Therapy for Danon Disease

January 22, 2019

- Phase 1 Clinical Trial to Commence in the Second Quarter of 2019 -

NEW YORK--(BUSINESS WIRE)--Jan. 22, 2019-- Rocket Pharmaceuticals, Inc. (Nasdaq: RCKT) (“Rocket”), a leading U.S.-based multi-platform gene therapy company, today announces the clearance of the Company’s Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) for RP-A501. RP-A501 is the Company’s first adeno-associated viral vector (AAV)-based gene therapy for the treatment of Danon disease that is in development under a collaboration with the University of California San Diego School of Medicine. UC San Diego Health will be the initial and lead center for the planned Phase 1 clinical trial under the direction of Eric Adler, M.D., Director of Cardiac Transplant and Mechanical Circulatory Support at UC San Diego Health and Professor of Medicine at University of California San Diego School of Medicine.

“This acceptance marks the first Rocket-sponsored IND cleared for our AAV platform and the third for our pipeline in only three months,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “RP-A501 represents the first investigational gene therapy for a monogenic heart failure syndrome and has the largest market opportunity for Rocket with an estimated 15,000 - 30,000 patients in the U.S. and E.U. We expect to initiate a Phase 1, first-in-human study in the U.S. in the second quarter of 2019 to assess the safety, tolerability and preliminary efficacy of RP-A501.”

“Danon disease is a devastating heart failure syndrome that develops during childhood and adolescence, and is followed by a rapid progression toward end-stage heart failure and death,” said Dr. Adler. “We look forward to advancing this potentially curative treatment option for the patients and families affected by this life-threatening disease.”

In parallel with the Phase 1 clinical trial, the Company plans to publish a comprehensive literature review of Danon cases and conduct a retrospective chart review. In addition, the Company will initiate a prospective natural history study with enrollment planned to commence in the first quarter of 2019.

About Danon Disease

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

About Rocket Pharmaceuticals, Inc.

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

Rocket Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding the safety, effectiveness and timing of product candidates that Rocket may develop, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe", "expect", "anticipate", "intend", "plan", "will give", "estimate", "seek", "will", "may", "suggest" or similar terms, variations of such terms or the negative of

those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, Rocket's ability to commence a registrational study in FA within the projected time periods, the potential advantages of Rocket's product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2017. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP, Corporate Strategy and IRO
Rocket Pharma, Inc.
The Empire State Building, Suite 7530
New York, NY 10118
www.rocketpharma.com
investors@rocketpharma.com