Rocket Pharmaceuticals Announces Patient Dosing Has Commenced for Phase 1 Clinical Trial of RP-A501, the First Gene Therapy to Treat a Monogenic Heart Failure Syndrome

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NEW YORK--(BUSINESS WIRE)--Jun. 18, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces that patient dosing has commenced in the open-label, Phase 1 clinical trial of RP-A501, the Company's adeno-associated viral vector (AAV)-based gene therapy for the treatment of Danon disease. University of California San Diego (UCSD) Health is the initial and lead center for the Phase 1 clinical trial under the leadership 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 and Barry Greenberg, M.D. Dr. Greenberg is the Director of the Advanced Heart Failure Treatment Program at UC San Diego Health and Professor of Medicine at UC San Diego School of Medicine, and is principal investigator of the trial.

"The initiation of patient dosing in our Phase 1 trial is a significant milestone for our RP-A501 program, the first investigational gene therapy for a monogenic heart failure syndrome," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Danon disease is a rapidly progressive cardiomyopathy where we believe gene therapy can make a meaningful difference in patient outcomes. Current standards of care for Danon disease, including heart transplant, are not curative and are associated with considerable morbidity and mortality. As a result, median survival for male Danon disease patients has been reported at age 19, caused by progressive heart failure. This underscores the urgent need for new treatment options like RP-A501 gene therapy for the patients and families contending with this debilitating, fatal disease."

"The advancement of RP-A501 into the clinic is a monumental step forward for the treatment of this devastating disease and, more broadly, the treatment of rare cardiac disorders," said Dr. Adler. "The team at UC San Diego Health is pleased to be the initial and lead center for the Phase 1 clinical trial of RP-A501 and we look forward to rapidly advancing it through the clinic on behalf of patients and families in need."

The non-randomized, open-label Phase 1 trial is expected to enroll 12-24 pediatric and young adult male patients. Two dose levels will be investigated in four patient cohorts separated by pediatric and adult age groups. The first cohort will receive a low dose level of $6.7x10^{13}$ genome copies/kg. Upon completion of patient dosing at the low dose, the Company plans to move to a higher dose. The study is designed to assess the safety and tolerability of a single infusion of RP-A501. Additional outcome measures include cardiomyocyte and skeletal muscle transduction by gene expression, histologic correction via endomyocardial biopsy, and clinical stabilization via cardiopulmonary testing. Further information about the clinical program is available here.

About Danon Disease

Danon disease is caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the European Union. The disease is often fatal in male patients in the second or third decade of life due to rapidly progressive heart failure. Available therapies for Danon disease include cardiac transplantation, which is associated with substantial complications and is 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 first two clinical programs are 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, and an AAV-based gene therapy for Danon disease, a devastating, pediatric heart failure condition. Rocket's pre-clinical pipeline programs for bone marrow-derived disorders are for Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). 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, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon disease, 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 pre-clinical and clinical results for its product candidates, which may not support further development and marketing approval, 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, 2018. 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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