Rocket Pharmaceuticals Announces Presentations at the American College of Cardiology 2019 Annual Meeting

March 15, 2019

Preclinical Data of RP-A501 in Danon Disease Demonstrates Restoration and Durable Prevention of Cardiac, Muscle and Liver Structure and Function

NEW YORK--(BUSINESS WIRE)--Mar. 15, 2019-- Rocket Pharmaceuticals, Inc. (Nasdaq:RCKT) (“Rocket”), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces one oral poster presentation and two poster presentations at the upcoming American College of Cardiology 2019 Annual Meeting being held March 16-18, 2019 in New Orleans, LA. 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 (UCSD). UCSD Health will be the initial and lead center for the planned Phase 1 clinical trial. Barry Greenberg, M.D., Director of the Advanced Heart Failure Treatment Program at UC San Diego Health and Professor of Medicine at University of California San Diego School of Medicine, will be the principal investigator.

“Danon is a disease of deficient autophagy caused by mutations in the gene for LAMP-2 that leads to death in adolescence and young male adulthood. Insights from literature including UCSD’s recently published natural history retrospective demonstrate that male patients have severe disease progression, leading to death at a median age of 19-21 years. Heart transplant and other interventions are not curative. Women have a delayed onset of cardiac symptoms, but may present with equally severe cardiomyopathy and suffer life-threatening complications. Global natural history studies are underway to expand upon these findings and inform trial design,” said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President, Clinical Development.

Dr. Schwartz continued, “Preclinical efficacy studies of RP-A501 have been conducted in LAMP-2 knockout mice at 2 different ages, one representing a young adolescent male before the onset of significant cardiac disease and the other representing an older male with established symptoms of Danon disease. These studies demonstrate both reversal and durable prevention of cardiac, muscle and liver dysfunction.”

“RP-A501 is the first investigational gene therapy for Danon disease and has the potential to revolutionize the treatment of this devastating cardiomyopathy. We are excited to move RP-A501 into the clinic in the second quarter of 2019,” concluded Dr. Schwartz.

Posters will be available on the Company’s website at the conclusion of the presentation time at: www.rocketpharma.com/pipeline/.

Oral Poster Presentation:

Title: AAV9.LAMP-2B Improves Metabolic and Physiologic Function in Murine and Human In-Vitro Models of Danon Disease
Session: 1048 - Heart Failure and Cardiomyopathies: Cutting Edge Basic Science Reports
Date: March 18, 2019
Time: 10:00 – 10:10 a.m. Central Time
Location: Heart Failure and Cardiomyopathies Moderated Poster Theater, Poster Hall, Hall F

Posters:

Title: Insights From Global Registry For Danon Disease Highlighting Differences Between Men and Women
Session: Heart Failure and Cardiomyopathies: Clinical 4
Date: March 17, 2019
Time: 3:45 – 4:30 p.m. Central Time
Location: Poster Hall, Hall F

Title: Longitudinal Echocardiographic Findings of Danon Disease: Insights From a Global Registry
Session: Heart Failure and Cardiomyopathies: Clinical 5
Date: March 18, 2019
Time: 9:45 – 10:30 a.m. Central Time
Location: Poster Hall, Hall F
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 patients in the second or third decade of life due to progressive heart failure unless treated with a cardiac transplantation, which is associated with numerous 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 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. Rocket’s additional pipeline programs for bone marrow-derived disorders are for 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 a devastating, pediatric heart failure indication, Danon 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.

Source: Rocket Pharmaceuticals, Inc.

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