Rocket Pharmaceuticals Presents Preclinical Data of RP-A501 for Danon Disease at the American Society of Gene and Cell Therapy 2019 Annual Meeting

May 2, 2019

- Biodistribution of RP-A501 Demonstrates High Concentration in Heart, the End-Organ Target in Danon Disease -

- Phase 1 Clinical Trial to Begin in Second Quarter of 2019 -

NEW YORK--(BUSINESS WIRE)--May 2, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today presents preclinical data of RP-A501 at the American Society of Gene and Cell Therapy 2019 Annual Meeting in Washington, D.C. RP-A501 is the Company's adeno-associated viral vector (AAV)-based gene therapy for the treatment of Danon disease. The data is included in an oral presentation by Annahita Keravala, Ph.D., Associate Vice President, AAV Platform, entitled, "Systemic Delivery of AAV9.LAMP2B for the Treatment of Danon Disease: Toxicology Studies in Mice and Cynomolgus Monkeys."

"Additional preclinical RP-A501 data continue to augment the evidence regarding this vector's potential to prevent, reduce, or reverse cardiac dysfunction. RP-A501 conferred high vector copy number (VCN) and LAMP2 protein expression in all four heart chambers, suggesting optimal tropism and uptake of the gene therapy. Specifically, VCNs in the ~10 range were about ten-fold higher in heart chambers versus skeletal muscle and most central nervous system tissues. Importantly, protein expression in all four heart chambers was higher in treated non-human primates versus wild type; this differential was most pronounced in cardiac muscle. Transduction and expression in the heart is critically important for Danon disease patients because heart failure is the overwhelming cause of mortality," said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President, Clinical Development of Rocket.

Investigational New Drug application (IND)-enabling toxicology studies were conducted in wild-type mice and non-human primates. Three dose levels were tested in mice, including 3×10^{13} vg/kg, 1×10^{14} vg/kg, and 3×10^{14} vg/kg. The highest dose level from the murine study, 3×10^{14} vg/kg, was tested in non-human primates. No dose-related adverse events were observed at all tested doses in both mice and non-human primates. Vector genomes, mRNA and protein expression were widely distributed across key tissues with high levels of transduction, transcription and translation detected in the heart, skeletal muscle, diaphragm and liver.

The ASGCT presentation also highlights previously reported preclinical efficacy data of RP-A501 in LAMP-2 knockout (KO) mice which showed dose-dependent improvements and restoration of cardiac function, with responses observed in both older and younger KO mice.

Full results from the ASGCT presentation will be available online at the conclusion of the oral presentation: https://www.rocketpharma.com/asgct-presentations/

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 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), 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 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, 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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