Rocket Pharmaceuticals Announces Preclinical Data for AAV-Based RP-A501, the First Investigational Gene Therapy Program for a Monogenic Heart Failure Syndrome

November 26, 2018

- Preclinical Data Package Shows Dose-Dependent Survival, Functional, Structural, and Molecular Benefits with No Safety or Tolerability Issues Observed -

- RP-A501's Market Opportunity: 15,000-30,000 Estimated Prevalence in the U.S. and E.U. -

- RP-A501 Anticipated to Enter the Clinic in the First Half of 2019; Strong Intellectual Property Protection in Place -

NEW YORK--(BUSINESS WIRE)--Nov. 26, 2018-- Rocket Pharmaceuticals, Inc. (Nasdaq:RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, today announced it is developing its first adeno-associated viral vector (AAV)-based gene therapy, RP-A501, that is designed to restore the lysosome-associated membrane glycoprotein 2 (*LAMP-2*) gene which is defective in patients afflicted with Danon disease. Rocket also announced animal study data which provides preclinical proof-of-concept for the RP-A501 program.

RP-A501 is in development under a collaboration led by Dr. Eric Adler, 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.

"Danon disease is a devastating multisystemic disorder that typically leads to death in patients from progressive heart failure in their teens and twenties. Along with severe cardiomyopathy, other Danon disease symptoms can include skeletal muscle weakness, liver disease, and intellectual impairment. Heart transplantation is an important treatment option but is not curative and is associated with approximately 50% ten-year survival post-transplant, considerable morbidity, and a very high cost to the medical system. Due to its nonspecific clinical presentation and the lack of standardized genetic testing, Danon disease is poorly recognized, under diagnosed, and often confused with other multisystemic disorders," said Dr. Adler. "RP-A501 is the first investigational gene therapy for Danon disease and has the potential to prevent and treat heart failure and other sequelae for these patients. Preclinical studies show RP-A501 improves survival and corrects the disease phenotype with dose-dependent improvements in molecular, structural, and functional endpoints alongside a clean safety and tolerability profile. Rocket has exceptional core strength in clinical development, and I look forward to advancing the first gene therapy program for a monogenic heart failure syndrome to the finish line with them."

"We are excited to announce this monogenic heart failure program as part of our growing pipeline. Our goal at Rocket is to develop paradigm-shifting medicines that meaningfully improve lives of patients with devastating diseases where no therapies exist," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Danon disease is a large opportunity for Rocket with an estimated prevalence of about 15,000-30,000 patients in the U.S. and E.U. Recognition of this disorder is growing rapidly with increasing acceptance of genetic testing and screening by cardiologists and other physicians since heart failure is often diagnosed without any underlying genetic screens. Natural History Studies are underway to support this market research."

"We have adequate vector supply for the planned clinical trials with RP-A501 and are on track to begin studies in early 2019. Finally, through an agreement with REGENXBIO we have gained rights for the AAV9 capsid as well as alternate capsids relevant for the devastating manifestations of Danon disease," said Dr. Shah.

Summary Preclinical RP-A501 Data:

Preclinical efficacy studies were performed in LAMP-2 knockout (KO) mice. Four doses of vector were tested for optimal transduction of the heart, skeletal muscle, and liver. Toxicology studies were conducted in wild-type mice and non-human primates. The results from these studies are summarized as follows:

- Increased survival rates were observed at higher doses of RP-A501 along with dose-dependent improvements and restoration of cardiac function.
- RP-A501 elicited phenotypic reversals at a structural and molecular level in cardiac, liver, and skeletal muscle tissue.
- There were no treatment-related adverse events or deaths associated with RP-A501. All doses were observed to be well-tolerated in Good Laboratory Practice (GLP) biodistribution and toxicology studies in both wildtype mice and additional studies in non-human primates.

Based on the preclinical safety and efficacy data observed in mice and non-human primate studies, Rocket plans to initiate a Phase 1 dose-escalation study of RP-A501 in patients with Danon disease in the first half of 2019.

Full preclinical data have been submitted to a medical conference.

Intellectual Property:

Rocket's intellectual property protection includes the exclusive development and commercialization rights for an AAV-based gene therapy treatment for Danon disease licensed from UCSD, as well as exclusive worldwide rights to the AAV9 capsid for the treatment of the indication through a license agreement with REGENXBIO.

A replay of the Company's conference call and webcast to discuss these preclinical results will be available on the "Investors" section of the Company's website at www.rocketpharma.com. The webcast replay will be available for a limited time.

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 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, 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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