Rocket Pharmaceuticals Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for RP-A501 Gene Therapy for Danon Disease

February 7, 2023

CRANBURY, N.J.--(BUSINESS WIRE)--Feb. 7, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-A501, the Company's investigational adeno-associated virus (AAV)-based gene therapy for the treatment of Danon Disease, a devastating and fatal genetic cardiac disease for which there are no disease-altering therapies available. RMAT designation was granted based on positive safety and efficacy data from the Phase 1 RP-A501 clinical trial and will provide the benefits of added intensive FDA guidance and expedited review through the program's development.

"Today's exciting RMAT designation demonstrates recognition from the FDA of the early meaningful benefit of RP-A501 in Danon Disease and its potential to deliver lifesaving treatment for patients," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "We look forward to the FDA's added guidance and support on the most efficient development and approval pathway for RP-A501, including on our anticipated Phase 2 pivotal trial with the opportunity for accelerated approval."

Dr. Shah continued, "RP-A501 is the first cardiac gene therapy to receive RMAT designation from the FDA, and today's news is another important step forward both for patients with Danon Disease and for the gene therapy field. We remain on track to initiate our Phase 2 trial in the second quarter and are thankful to the FDA for their continued collaboration."

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising pipeline products, including gene therapies. A regenerative medicine therapy is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or therapy has the potential to address unmet medical needs for such disease or condition.

Similar to Breakthrough Therapy designation, RMAT designation provides the benefits of intensive FDA guidance on efficient drug development, including the ability for early FDA interactions to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the Biologics License Application (BLA) and other opportunities to expedite development and review. Rocket also holds Orphan Drug (U.S.) and Rare Pediatric designations for the RP-A501 program.

The initiation of the Phase 2 pivotal trial is on track for the second quarter of 2023. As previously disclosed, the Company anticipates pursuing a single arm, open-label trial with a biomarker-based composite endpoint and a natural history comparator.

Results from the Phase 1 program represent one of the most comprehensive investigational gene therapy datasets for any cardiac condition. RP-A501 was generally well tolerated with evidence of restored expression of the deficient LAMP2 protein and durable improvement or stabilization of clinical parameters in the Danon Disease patients treated in the Phase 1 study.

Data show consistent and robust improvements in multiple parameters including protein expression, reduced autophagic vacuoles, brain natriuretic peptide (BNP), troponin, left ventricular (LV) mass and thickness, and improved New York Heart Association (NYHA) class and Kansas City Cardiomyopathy Questionnaire (KCCQ/Quality of Life) measurements. Notably, these improvements and stabilization of BNP and NYHA class are in stark contrast to BNP increases and NYHA class deterioration observed in a representative sample of pediatric and adolescent natural history patients. Overall, the results demonstrate improvements and/or normalization across multiple quantifiable parameters that cardiologists use in clinical practice to enable risk assessment and treatment decisions.

About Danon Disease

Danon Disease is a rare X-linked inherited disorder caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. This results in accumulation of autophagosomes and glycogen, particularly in cardiac muscle and other tissues, which ultimately leads to heart failure, and for male patients, frequent death during adolescence or early adulthood. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and Europe. The only available treatment option for Danon Disease is 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) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare childhood disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (LVV)-based gene therapy are for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, and Pyruvate Kinase Deficiency (PKD), a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. Rocket also has preclinical AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and BAG3-associated dilated cardiomyopathy (DCM). 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 and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its Danon Disease program, including its planned pivotal trial, 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 monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, 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 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, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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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Media
Kevin Giordano
kgiordano@rocketpharma.com

Investors
Brooks Rahmer
investors@rocketpharma.com

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