Rocket Pharmaceuticals Announces FDA Lifts Clinical Hold on Danon Disease Trial of RP-A501

August 16, 2021

—Initiation of Pediatric Patient Dosing of RP-A501 Expected in Q3 —

—Comprehensive Clinical Update from Phase 1 Clinical Trial Planned for Q4 —

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 16, 2021-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold on the Company's Phase 1 clinical trial of RP-A501 for the treatment of Danon Disease, allowing patient enrollment to resume. The hold was removed after the Company addressed the FDA's requests to modify the trial protocol and other supporting documents with revised guidelines for patient selection and management. The Company has initiated steps to resume the program as soon as possible and expects to commence dosing in the low-dose (6.7e13 vg/kg) pediatric patient cohort in the third quarter.

"We are grateful for the collaboration between the FDA and our team in reaching agreement on protocol updates allowing us to resume patient enrollment in our Danon Disease trial. We look forward to progressing this critical work on behalf of all Danon patients," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "We are moving as quickly as possible to resume dosing and commence treatment this quarter. Additionally, given the activity observed among young adults in our low-dose cohort, in agreement with the FDA, we are now proceeding with the pediatric cohort. This is another important step forward as we believe the pediatric Danon population has the potential to realize the maximum benefit from our Danon Disease gene therapy program. We continue to anticipate reporting updated longer-term data from the low-dose (6.7e13 vg/kg) and higher-dose (1.1e14 vg/kg) young adult cohorts in the fourth quarter."

Rocket's Danon Disease program was placed on clinical hold by the FDA in May of 2021 to modify the study protocol and other supporting documents with revised guidelines for patient selection and safety management. No new drug-related safety events were observed in the low-dose or higher-dose young adult cohorts as part of the clinical hold or during the hold.

The non-randomized, open-label Phase 1 trial is designed to enroll both pediatric and young adult male patients to assess the safety and tolerability of a single intravenous (IV) 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 cardiac imaging and functional cardiopulmonary testing.

About RP-A501

RP-A501 is an investigational gene therapy product being developed for Danon Disease and the first potential gene therapy for monogenic heart failure. It consists of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a functional version of the human *LAMP2B* transgene (AAV9.*LAMP2B*). RP-A501 is currently being evaluated in an ongoing Phase 1 clinical trial, in which the Company observed preliminary activity data of RP-A501 as well as a favorable tolerability profile.

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 many male patients, 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 the 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) ("Rocket") is advancing an integrated and sustainable pipeline of genetic therapies that aim to correct the root cause of complex and rare childhood disorders. The company's platform-agnostic approach enables it to design the best potential 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 potential 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, Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia and Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. Rocket's first clinical

program using adeno-associated virus (AAV)-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. 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 its guidance for 2021 in light of COVID-19, 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), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold 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, 2020, filed March 1, 2021 with the SEC. 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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