Rocket Pharmaceuticals Announces First Patient Treated in Higher Dose Cohort in Phase 1 Clinical Trial of RP-A501 for Danon Disease

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—Safety Data from Low Dose Cohort Supports FDA Clearance for Higher Dose Patient Treatment—

—Phase 1 Preliminary Safety and Efficacy Data Expected Fourth Quarter 2020—

NEW YORK--(BUSINESS WIRE)--Sep. 2, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare pediatric disorders, today announces it has treated the first patient in the higher dose cohort in its open-label, Phase 1 clinical trial of RP-A501, the Company's adeno-associated viral vector (AAV)-based gene therapy for the treatment of Danon Disease. Treatment of the higher dose cohort comes after successful completion of the low dose cohort and clearance from the U.S. Food and Drug Administration (FDA) and Independent Data Safety Monitoring Committee (IDSMC). The second cohort of the Phase 1 study evaluates RP-A501 at a higher dose level of 1.1×10^{14} genome copies/kilogram in male patients 15 years of age and older. The Phase 1 study will assess the safety, tolerability and preliminary efficacy of RP-A501.

"Today marks an important milestone in the development of RP-A501 for Danon Disease, the first investigational gene therapy in development for the treatment of inherited heart failure," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "RP-A501 represents a potentially holistic and transformative approach to Danon Disease, a devastating disease often resulting in end-stage heart failure and early mortality. The only definitive treatment option available for this multisystemic disorder is heart transplantation, which is associated with a 50% mortality rate during the decade following transplant and does not address the full spectrum of the disease course. Our gene therapy approach, utilizing RP-A501, aims to address all manifestations associated with Danon Disease including cardiomyopathy, skeletal myopathy and intellectual disability. We look forward to progressing this trial and enabling a potentially curative treatment option for this devastating disease that affects so many lives."

The non-randomized, open-label Phase 1 trial aims to enroll both pediatric and young adult male patients in escalating dose cohorts. Following the review of safety data from the first cohort, all subsequent cohorts will include 2-4 patients per cohort, adjusted down from 3-6 patients in the original protocol. The study is designed 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 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 advancing an integrated and sustainable pipeline of genetic therapies that 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, 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 2020 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop, 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 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 when clinical trial sites will resume normal business operations, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, filed August 5, 2020 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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