Rocket Pharmaceuticals Presents Clinical Data from RP-A501 Trial in Danon Disease at the Heart Failure Society of America (HFSA) Annual Scientific Meeting 2021

September 14, 2021

—Previously disclosed positive data from the low-dose (6.7e13 vg/kg) cohort of the ongoing Phase 1 trial presented at HFSA's Annual Scientific Meeting 2021 —

—Data demonstrated RP-A501 was well tolerated and showed increasing and durable benefit —

—Low-dose pediatric patient cohort expected to commence in Q3; full update anticipated Q4 —

CRANBURY, N.J.--(BUSINESS WIRE)--Sep. 14, 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 an oral presentation of previously disclosed data from the ongoing Phase 1 trial of RP-A501 for the treatment of Danon Disease was given at the Heart Failure Society of America (HFSA) Annual Scientific Meeting 2021. The presentation overviewed data from the low-dose (6.7e13 vg/kg) cohort which demonstrated the investigational gene therapy RP-A501 was well tolerated and showed progressive and durable clinical benefit.

"Patients with Danon Disease desperately need a treatment option that provides meaningful therapeutic benefit with a manageable safety profile, and we are excited to be a part of the HFSA Annual Meeting and to share this important work," said Jonathan Schwartz, M.D., chief medical officer of Rocket Pharma. "We continue to be encouraged by the safety and durable clinical benefit RP-A501 has demonstrated in the low dose, with one patient now followed to 24 months. Additionally, we are working expeditiously to commence treatment in the low-dose (6.7e13 vg/kg) pediatric patient cohort in the third quarter and expect to provide a comprehensive clinical update from the Phase 1 trial in the fourth quarter."

Data from the Low-Dose Adult Cohort (n=3) of the Phase 1 Trial of RP-A501 in Danon Disease

- RP-A501 was generally well tolerated with a manageable safety profile. All observed adverse effects were transient and reversible with no lasting clinical sequelae. Steroid-induced myopathy was observed in two of the three patients >2 weeks after dosing, which also resolved.
- All three low-dose patients demonstrated evidence of sustained cardiac *LAMP2B* expression by Western blot and/or immunohistochemistry with decreased vacuoles and improved tissue architecture on electron microscopy.
- All three patients demonstrated improvements in the 6 minute walk test (6MWT). One patient improved from a pretreatment baseline of 443 meters (m) to 467 m at 24 months. The second patient improved from a pretreatment baseline of 405 m to 410 m at 18 months. The third patient improved from a pretreatment baseline of 427 m to 435 m at 15 months.
- An improvement in NYHA class in two of the three low-dose patients was observed. In these two patients, a substantial improvement of a key marker of heart failure, B-type natriuretic peptide (BNP), was also observed. BNP decreased from a pretreatment baseline by 75 percent in one patient and 79 percent in the other. The third patient has demonstrated stabilization of NYHA class and BNP.

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, from which preliminary data of the low-dose cohort showed it was generally well tolerated and provided evidence of improved cardiac function in patients.

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) (“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 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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