



## Rocket Pharmaceuticals Announces Upcoming Danon Disease Presentation at the Heart Failure Society of America Annual Scientific Meeting 2021

September 10, 2021

—Oral presentation on previously disclosed data from the ongoing Phase 1 trial of RP-A501—

CRANBURY, N.J.--(BUSINESS WIRE)--Sep. 10, 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 previously disclosed data from the ongoing Phase 1 trial of RP-A501 in Danon Disease will be presented in a late-breaking oral presentation at the upcoming Heart Failure Society of America (HFSA) Annual Scientific Meeting 2021. The meeting will take place September 10-13, 2021 at the Gaylord Rockies Hotel and Conference Center in Aurora, Colorado.

Details for Rocket's oral presentation are as follows:

**Title:** Results From First-In-Human Clinical Trial Of RP-A501 (AAV9:LAMP2B) Gene Therapy Treatment For Danon Disease

**Session Title:** Late Breaking Clinical Trials

**Session Date:** Monday, September 13, 2021

**Presentation Time:** 11:00 a.m. - 12:00 p.m. MDT

**Presenter:** Barry Greenberg, MD, FHFSA, Distinguished Professor of Medicine at UC San Diego School of Medicine and director of the Advanced Heart Failure Treatment Program at the Cardiovascular Institute at UC San Diego Health

### 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 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 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](http://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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