Rocket Pharmaceuticals Receives European Medicines Agency (EMA) Priority Medicines (PRIME) Designation for RP-A501 Gene Therapy for Danon Disease

May 31, 2023

CRANBURY, N.J.--(BUSINESS WIRE)--May 31, 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 European Medicines Agency (EMA) has granted Priority Medicines (PRIME) 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 inherited cardiomyopathy for which there are no curative therapies. PRIME designation was granted based on positive safety and efficacy data from the Phase 1 clinical trial of RP-A501 in patients with Danon Disease and the potential of RP-A501 to meet the high unmet medical need in this population.

PRIME designation offers the benefits of early and enhanced support from the EMA for the development of medicines that target unmet medical needs, as well as the opportunity for an accelerated review of the marketing application. Rocket was also recently granted Regenerative Medicine Advanced Therapy (RMAT) designation for its RP-A501 program, which also holds Fast Track, Orphan Drug (U.S.) and Rare Pediatric designations.

"PRIME designation from the EMA further highlights the positive benefit/risk profile of RP-A501 in addressing the critical unmet need of patients facing Danon Disease," said Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer, Rocket Pharma. "We are thrilled by the opportunity that PRIME grants us, so that we may collaborate with our European Regulatory partners on the development of RP-A501 in the most expedient and efficient path forward."

Results from the Phase 1 trial represent one of the most comprehensive investigational gene therapy datasets for any cardiac condition. RP-A501 was associated with a favorable safety profile. The data demonstrated consistent and robust improvements in multiple clinical and highly relevant laboratory parameters including LAMP-2 protein expression, reduced autophagic vacuoles, brain natriuretic peptide (BNP), high sensitivity troponin I, and left ventricular mass and wall thickness. In addition, there was improvement in symptoms, as assessed by New York Heart Association class and quality of life, as measured by the Kansas City Cardiomyopathy Questionnaire. Notably, the improvements and stabilization of BNP in Phase 1 patients were in direct contrast to worsening patterns observed in patients enrolled in a concurrent, prospective natural history study. The results demonstrated improvements and/or normalization across multiple quantifiable parameters that cardiologists use in clinical practice to enable risk assessment and treatment decisions.

About EMA PRIME Designation

Priority Medicines (PRIME) designation was created by the European Medicines Agency (EMA) to enhance support for the development of innovative medicines that target an unmet medical need and demonstrate the potential to achieve relevant clinical outcomes on morbidity, mortality or underlying disease progression. The PRIME designation offers enhanced early interaction with companies developing promising medicines, to optimize development plans and speed up evaluation. PRIME focuses on medicines that may offer a major therapeutic advantage over existing treatments, or that benefit patients without treatment options.

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 is a high unmet medical need for patients with 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 "aim," "anticipate," "believe," "can," "continue," "design," "estimate," "expect," "intend," "may," "plan," "potential," "will give," "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, 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, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any 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, 2022, filed February 28, 2023 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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Source: Rocket Pharmaceuticals, Inc.