Rocket Pharmaceuticals Receives EMA Priority Medicines (PRIME) Designation for RP-L201 Gene Therapy for Treatment of Leukocyte Adhesion Deficiency-I

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— PRIME Designation to Facilitate Greater EMA Collaboration for Development and Potential Accelerated Regulatory Review in Europe —

— LAD-I Program Now Holds All Available Accelerated Regulatory Designations in the U.S. and EU —

CRANBURY, N.J.--(BUSINESS WIRE)--Mar. 29, 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 European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation to RP-L201, the Company's investigational gene therapy for Leukocyte Adhesion Deficiency-I (LAD-I). PRIME designation was granted based on encouraging preliminary safety and efficacy data from the ongoing Phase 1/2 clinical trial of RP-L201.

"We are delighted that the EMA has awarded PRIME designation to RP-L201 for the treatment of LAD-I. PRIME completes the full complement of U.S. and EU accelerated regulatory designations for RP-L201 and signals that regulators recognize the high unmet medical need in treating this devastating pediatric disease and our gene therapy's potential to address the root cause of this disorder," said Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer of Rocket. "More than half of LAD-I patients suffer with a severe variant in which mortality occurs in up to 75% of children prior to age two in the absence of a successful bone marrow transplant. Securing all possible accelerated designations will enable us to collaborate with both the FDA and EMA to speed the delivery of a potentially curative option for these patients. We look forward to sharing initial Phase 2 data from our potentially registration-enabling LAD-I trial in the second quarter of 2021."

The PRIME program aims to optimize development plans and speed up evaluation of medicines that may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options. These medicines are considered priority medicines by the EMA and are intended to reach patients earlier. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.

The ongoing, non-randomized, open-label Phase 1/2 study of RP-L201 recently completed enrollment. It is designed to evaluate the safety and efficacy of lentiviral vector (LVV)-based RP-L201 in pediatric patients with severe LAD-I, as defined by CD18 expression of less than 2%. Data from the study presented at the 62nd American Society of Hematology Annual Meeting demonstrate evidence of safety and efficacy in three pediatric patients with severe LAD-I. These patients have shown sustained CD18 expression exceeding the 4-10% threshold associated with survival into adulthood and similarly encouraging peripheral blood vector copy numbers. RP-L201 was well tolerated with no drug product safety issues reported with infusion or post-treatment. The study is being conducted at the University of California Los Angeles, University College London (UCL)/Great Ormond Street Children's Hospital, and Hospital Infantil Universitario Niño Jesús.

Further information about the RP-L201 clinical program is available here.

About RP-L201

RP-L201 is a gene therapy containing autologous (patient-derived) hematopoietic stem cells. The cells are genetically modified with a LVV to contain a functional copy of the *ITGB2* gene to treat LAD-I. Rocket holds FDA Regenerative Medicine Advanced Therapy, Rare Pediatric, and Fast Track designations in the U.S., PRIME and Advanced Therapy Medicinal Product designations in the EU, and Orphan Drug designation in both regions for the program. RP-L201 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras and Instituto de Investigación Sanitaria Fundación Jiménez Díaz. The LVV was developed in a collaboration between UCL and CIEMAT.

About Leukocyte Adhesion Deficiency-I

Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by mutations in the *ITGB2* gene encoding for the beta-2 integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. As a result, children with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Children who survive infancy experience recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia. Without a successful bone marrow transplant, mortality in

patients with severe LAD-I is 60-75% prior to the age of 2 and survival beyond the age of 5 is uncommon. There is a high unmet medical need for patients with severe LAD-I.

Rocket's LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (CIRM) (Grant Number CLIN2-11480). The contents of this press release are solely the responsibility of Rocket and do not necessarily represent the official views of CIRM or any other agency of the State of California.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) 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, 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 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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