Rocket Pharmaceuticals Receives FDA Regenerative Medicine Advanced Therapy Designation for RP-L201 Gene Therapy for Leukocyte Adhesion Deficiency-I

March 9, 2021

— FDA RMAT Designation for RP-L201 to Help Expedite Development and Registration —

— Enrollment Complete in Phase 1/2 Clinical Trial of RP-L201 for the Treatment of LAD-I —

CRANBURY, N.J.--(BUSINESS WIRE)--Mar. 9, 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 U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-L201, its investigational gene therapy for the treatment of Leukocyte Adhesion Deficiency-I (LAD-I). RMAT designation was granted based on encouraging preliminary safety and efficacy data from the ongoing Phase 1/2 clinical trial of RP-L201. Additionally, patient enrollment has been fully completed for the Phase 1/2 trial. 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.

"Receiving RMAT designation and completing Phase 1/2 patient enrollment are important steps in advancing our RP-L201 LAD-I program as efficiently and responsibly as possible," said Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer of Rocket. "We look forward to maximizing the opportunity for enhanced dialogue with the FDA as we work closely with the agency on potential registration, thanks to the RMAT designation. Importantly, completing Phase 1/2 patient enrollment against the backdrop of a global pandemic is a testament to our team, collaborators, and the patients participating in the trial. I am grateful to all of them for their unwavering commitment as we seek to address the life-threatening impact of LAD-I on the lives of many infants, young children, and their families. We look forward to sharing data from our LAD-I trial in the second quarter in addition to the remainder of the pipeline throughout 2021."

RMAT designation was established to help expedite the development and approval of regenerative medicine products, including cell and gene therapies. The designation is granted to an investigational product that intends to treat, modify, reverse or cure a serious or life-threatening disease or condition, and is supported by preliminary clinical evidence demonstrating its potential to address an unmet medical need. RMAT designation allows companies to work closely with the FDA on a program's development and includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designations. Rocket also holds Rare Pediatric (U.S.), Orphan Drug (U.S./EU), and Advanced Therapy Medicinal Product (EU) designations for the RP-L201 program.

The ongoing, non-randomized, open-label Phase 1/2 study of RP-L201 is designed to evaluate the safety and efficacy of the gene therapy 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 (ASH) 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.

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

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.

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 lentiviral vector was developed in a collaboration between UCL and CIEMAT.

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 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 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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