Rocket Pharmaceuticals Receives Funding from the California Institute for Regenerative Medicine (CIRM)

April 30, 2019

- $6.5mm Grant to Fund U.S. Phase I/II Leukocyte Adhesion Deficiency-I Registration-Enabling Study -

NEW YORK--(BUSINESS WIRE)--Apr. 30, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces that the California Institute for Regenerative Medicine (CIRM) has awarded Rocket a $6.5mm CLIN2 grant award to support the clinical development of gene therapy for Leukocyte Adhesion Deficiency-I (LAD-I). The CIRM was founded in 2004 following the passing of Proposition 71 or the California Stem Cell Research and Cures Initiative, which allocated $3 billion in state funding for stem cell research conducted in California.

Rocket’s Investigational New Drug Application (IND) for RP-L201 was accepted by the U.S. Food and Drug Administration in November 2018. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled in the U.S. clinical site, University of California, Los Angeles Mattel Children’s Hospital, led by PI Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. The trial will evaluate the safety and efficacy of the infusion of autologous hematopoietic stem cells transduced with a lentiviral vector encoding the *ITGB2* gene.

“RP-L201 has the ability to address a significant unmet need in infants and children who suffer from recurrent and life-threatening infections. Pre-clinical data suggests RP-L201 can meaningfully increase CD18 expression and neutrophil migration and markedly increase potential survival,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “We are honored that CIRM has recognized the potential value of RP-L201 and we look forward to dosing our first patient in the months ahead.”

About Leukocyte Adhesion Deficiency-I

Severe leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by a mutation of the *ITGB2* gene that encodes 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 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 exceedingly rare. The unmet medical need for patients with severe LAD-I is therefore significant.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”) is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket’s multi-platform development approach applies the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Rocket’s additional pipeline programs for bone marrow-derived disorders are for Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket is also developing an AAV-based gene therapy program for a devastating, pediatric heart failure indication, Danon disease. 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, effectiveness and timing of product candidates that Rocket may develop, including in collaboration with academic partners, 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, Rocket's ability to commence a registrational study in FA within the projected time periods, the potential advantages of Rocket's product candidates, 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 and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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, 2018. 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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