Rocket Pharmaceuticals Sponsors Clinical Trial at University of California, Los Angeles (UCLA)

March 11, 2019

- Donald B. Kohn, M.D., of UCLA to lead U.S. clinical development efforts for Leukocyte Adhesion Deficiency-I and Infantile Malignant Osteopetrosis programs -

NEW YORK--(BUSINESS WIRE)--Mar. 11, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, today announces a research agreement to support the clinical development of Rocket's lentiviral vector (LVV)-based gene therapy programs in Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO) towards registrational trials. Rocket's LVV-based gene therapy program for LAD-I, RP-L201, is in clinical development with Rocket's European partners at the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT). The LVV-based gene therapy for IMO, RP-L401, is in preclinical development in Sweden with European partner Lund University.

UCLA and its Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research will serve as the lead U.S. clinical research center for the planned registrational clinical trial for LAD-I and also a lead U.S. clinical site for IMO. Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), and Molecular and Medical Pharmacology at UCLA, will serve as the principal investigator for the planned trials and will oversee the management of the clinical trial site. Additional terms of the agreement were not disclosed.

"Rocket has put the building blocks in place to execute upon our strategy to transform our pipeline with precision and exigency. Dr. Kohn was chosen to lead the LAD-I and IMO programs as he shared our passion for gene and cellular therapy research for rare diseases," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We look forward to initiating our first clinical trial for LAD-I in the coming months and advancing our IMO program towards the clinic in 2020."

Rocket's Investigation New Drug (IND) application for RP-L201 was cleared by the U.S. Food and Drug Administration (FDA) in 2018. The Company expects to initiate a Phase 1/2 clinical trial in support of registration in the second quarter of 2019. The Phase 1 portion of the trial will assess the safety and tolerability of RP-L201. The Phase 2 portion of the trial will evaluate overall survival. Further information is available here.

About Leukocyte Adhesion Deficiency-I

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 enables their extravasation from blood vessels to combat infections. The degree of CD18 deficiency determines the severity of the disease, which can be categorized as moderate or severe based on functional CD18 expression. Most patients have the severe form of the disease, with less than 2% of normal neutrophil CD18 expression. Severe LAD-I causes recurrent and life-threatening infections which are frequently fatal despite antibiotic use. Approximately 75% of patients die before age 2 unless an allogenic hematopoietic stem cell transplantation is performed.

About Infantile Malignant Osteopetrosis

Infantile Malignant Osteopetrosis (IMO) is a severe form of osteopetrosis most commonly caused by a genetic mutation of the TCIRG1 gene which leads to ineffective osteoclast function. Osteoclasts play a vital role in maintaining bone growth health by breaking down bone tissue through the bone resorption process and maintaining equilibrium with bone generation. Impaired bone resorption causes increased bone mass and density, skeletal deformities, debilitating neurological abnormalities and bone marrow failure. Symptoms are typically present in the first year of life and the disorder is frequently fatal within the first decade of life unless treated with an allogenic hematopoietic stem cell transplantation.

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.

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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