Rocket Pharmaceuticals Announces First Patient Treated in Phase 1/2 Registrational Trial of RP-L201 for LAD-I

September 5, 2019

- Preliminary Phase 1 Data Expected by the End of 2019 -

NEW YORK--(BUSINESS WIRE)--Sep. 5, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces that the first patient received investigational therapy in the open-label, Phase 1/2 clinical trial of RP-L201. RP-L201 is the Company’s lentiviral vector (LVV)-based gene therapy for the treatment of severe Leukocyte Adhesion Deficiency-I (LAD-I) that was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT: Madrid, Spain). The lentiviral vector was developed in a collaboration between The University College of London (UCL) and CIEMAT. The University of California, Los Angeles (UCLA) Mattel Children’s Hospital, is the lead U.S. clinical research center under the leadership of Donald B. Kohn, M.D. Dr. Kohn is Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, a member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA, and is principal investigator of the trial.

“We are excited to commence patient dosing in our Phase 1/2 trial of RP-L201 in support of registration, as it not only offers hope for very young patients and families affected by this devastating disease, but also a potential first path to approval for Rocket’s gene therapy platform,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “LAD-I represents a major area of unmet medical need as most severely-afflicted patients die before the age of 2 in the absence of a hematopoietic stem cell transplant, which is associated with graft-versus-host disease. Our hope is that patients would benefit from a one-time administration of autologous gene therapy facilitated with busulfan conditioning. This process does not require immunosuppression and eliminates the risk of graft-versus-host disease.”

The non-randomized, open-label Phase 1/2 trial is expected to enroll nine pediatric patients globally. The Phase 1 portion of the trial is expected to enroll two patients and will assess the safety and tolerability of RP-L201. The Phase 2 portion of the trial will evaluate overall survival at multiple sites globally (U.S. and E.U.). Further information about the clinical program is available here.

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 first two clinical programs using 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, and Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal. Rocket’s first clinical program using AAV-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. Rocket’s pre-clinical pipeline programs for bone marrow-derived disorders are for Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO). 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, 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 pre-clinical and clinical results for its product candidates, which may not support further development and marketing approval, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190905005285/en/

Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP, Strategy & Corporate Development
Rocket Pharma, Inc.
The Empire State Building, Suite 7530
New York, NY 10118
www.rocketpharma.com
investors@rocketpharma.com