Rocket Pharmaceuticals Announces IMPD Clearance of RP-L301 Gene Therapy for Pyruvate Kinase Deficiency

September 23, 2019

–Rocket's Largest Lentiviral Pipeline Opportunity with an Estimated Prevalence of 3,000 to 8,000 Patients in the U.S. and EU–

–Phase 1 Clinical Trial to Commence in the Fourth Quarter–

NEW YORK--(BUSINESS WIRE)--Sep. 23, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces Investigational Medicinal Product Dossier (IMPD) clearance from the Spanish Agency for Medicines and Health Products (AEMPS) for RP-L301. RP-L301 is the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Pyruvate Kinase Deficiency (PKD) that was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria Fundación Jiménez Díaz (IIS-FJD).

"We are excited to advance the first gene therapy for PKD to the clinic following the clearance of the IMPD from the AEMPS for RP-L301," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "This news is particularly timely for Rocket as it follows our sponsorship and participation in the Patient Focused Drug Development Meeting on PKD held by NORD last week, as well as the 3rd Annual PKD Patient's Forum in May. At these events, we gained insights into the impact of PKD on the daily life of patients, and the challenges of current therapies. We continue to be highly committed to working closely with patients, families and physicians in addressing many of these issues with a potentially curative gene therapy option."

"In preclinical models, RP-L301 reverses the hemolytic phenotype, including normalization of splenomegaly and reduction of anemia and reticulocytosis, when at least 20-30% of bone marrow progenitor cells are genetically corrected," said Dr. José- Carlos Segovia, scientific leader of the project and PKD Scientific Advisor. "We look forward to investigating the potential of this new, single-administration treatment option for patients who are desperately in need of promising and potentially definitive therapy."

The planned open-label, single-arm, Phase 1 clinical trial of RP-L301 will enroll a total of six adult and pediatric transfusion-dependent PKD patients in Europe and the U.S. The trial will be separated into three cohorts of older pediatric, younger pediatric and adult age groups. Upon completion of an initial adult cohort, the Company plans to move to the pediatric cohorts. The trial is designed to assess the safety, tolerability and preliminary efficacy of RP-L301. Hospital Infantil Universitario Niño Jesús will serve as the lead site in Europe for pediatrics and Hospital Universitario Fundación Jiménez Díaz will serve as the lead site in Europe for adult patients.

The Patient Focused Drug Development (PFDD) Meeting on PKD last week provided patients, families and caregivers the opportunity to share their personal experiences living with the disease and their familiarities with currently available treatments. This event was organized by the National Organization for Rare Disorders (NORD) and the Foundation for Rare Blood Diseases (SZB). Sponsoring the PKD PFDD Meeting is part of Rocket's commitment to promoting rare disease education and awareness. Additional initiatives include the 3rd Annual PKD Patients' Forum in Madrid, Spain, and a Rocket-sponsored Rare Disease Day educational event on February 28 to show support for those living with rare diseases.

About Pyruvate Kinase Deficiency

Pyruvate Kinase Deficiency (PKD) is a rare, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red cell destruction and the disorder ranges from mild to life-threatening anemia. PKD has an estimated prevalence of 3,000 to 8,000 patients in the United States and the European Union. Children are the most commonly and severely affected subgroup of patients. Currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload.

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 preclinical 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.

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