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

October 22, 2019

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

–Precedent-Setting Global Phase 1 Study with Commercial Manufacturer is Now Enrolling–

NEW YORK--(BUSINESS WIRE)--Oct. 22, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces the clearance of the Company's Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) 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). The IND acceptance follows the recent clearance of the Investigational Medicinal Product Dossier (IMPD) for RP-L301 by the Spanish Agency for Medicines and Health Products (AEMPS) in September. The trial will be led by principal investigator Sandeep Soni, M.D., Clinical Associate Professor of Stem Cell Transplantation and Regenerative Medicine at the Stanford University School of Medicine.

"RP-L301 represents the first gene therapy candidate in development for PKD, a hematologic disorder in which the current treatment options, chronic blood transfusions and splenectomy, are associated with burdensome side effects and end-organ damage," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Promising preclinical RP-L301 data suggest correction of multiple key disease elements, including hemoglobin increases, reticulocyte reduction, and correction of splenomegaly. We look forward to advancing this potentially curative gene therapy into the clinic in the fourth quarter on behalf of the pediatric and adult PKD patients and families in need."

"In less than 12-months, four Rocket-sponsored INDs received clearance from the FDA. The RP-L301 IND marks an important milestone as it is the first global Phase 1 study for Rocket in the U.S. and EU. This achievement would not have been possible without the team's dedication and commitment to bringing first and best in class curative gene therapies to patients as quickly as possible," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development of Rocket.

The global Phase 1 open-label, single-arm, clinical trial will enroll six adult and pediatric transfusion-dependent PKD patients in the U.S. and Europe. The trial will be comprised of three cohorts to assess RP-L301 in young pediatric, older pediatric and adult populations. Rocket intends to complete the adult cohort before moving into pediatric patients. The trial is designed to assess the safety, tolerability and preliminary efficacy of RP-L301. Lucile Packard Children's Hospital Stanford will serve as the lead site in the U.S. for adult and pediatric patients. 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.

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 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, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, and Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. 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 program is for Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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.

Source: Rocket Pharmaceuticals, Inc.

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