Rocket Pharmaceuticals Announces First Patient Treated in Phase 1 Trial of RP-L301 Gene Therapy for Pyruvate Kinase Deficiency

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—Preliminary Phase 1 Data Anticipated in the Fourth Quarter—

—Rocket's Largest Lentiviral Pipeline Program Addresses Unmet Need for an Estimated 3,000 to 8,000 Patients in the U.S. and Europe—

NEW YORK--(BUSINESS WIRE)--Jul. 13, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces that the first patient has received investigational therapy in the open-label, Phase 1 clinical trial of RP-L301, the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Pyruvate Kinase Deficiency (PKD), a rare monogenic red blood cell disorder.

"We are pleased to have treated the first patient in our Phase 1 trial of RP-L301, marking an important step forward in addressing a high unmet need for new therapies," said Jonathan D. Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. "PKD is a genetic disorder characterized by red cell destruction and anemia that can be severe or even life-threatening. Children are often most severely affected, and the current treatment options—chronic transfusions and splenectomy—are associated with burdensome side effects including iron overload and end-orgar damage. We believe gene therapy treatment with RP-L301 has the potential to be a safe and transformative approach to improve long-term patient outcomes."

The global Phase 1 open-label, single-arm, clinical trial is expected to 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 (age 8-11), older pediatric (age 12-17) and adult populations. The trial is designed to assess the safety, tolerability and preliminary efficacy of RP-L301, and initial safety evaluation will occur in the adult cohort before evaluation in pediatric patients. Lucile Packard Children's Hospital Stanford is the lead site in the U.S. for adult and pediatric patients. Hospital Infantil Universitario Nino Jesus is the lead site in Europe for pediatrics and Hospital Universitario Fundacion Jimenez Diaz is the lead site in Europe for adult patients. Further information about the clinical program is available here.

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.

RP-L301 was in-licensed from the Centro de Investigaciones Energeticas, Medioambientales y Tecnologicas (CIEMAT), Centro de Investigacion Biomedica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigacion Sanitaria Fundacion Jimenez Diaz (IIS-FJD).

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") is advancing an integrated and sustainable pipeline of genetic therapies that correct the root cause of complex and rare disorders. The company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (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, Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia and Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. 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 its guidance for 2020 in light of COVID-19, 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 monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding when clinical trial sites will resume normal business operations, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, 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 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-Q for the quarter ended March 31, 2020, filed May 8, 2020 with the SEC. 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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Claudine Prowse, Ph.D.
SVP, Strategy & Corporate Development
investors@rocketpharma.com

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