Rocket Pharmaceuticals Sponsors 3rd Annual PKD Patients' Forum

May 29, 2019

NEW YORK--(BUSINESS WIRE)--May 29, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today sponsors the 3rd Annual Pyruvate Kinase Deficiency (PKD) Day in Madrid, Spain, a day devoted to patients, families, clinicians and global scientific experts. This event is co-sponsored by Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER), Instituto de Investigación Sanitaria Fundación Jiménez Díaz (IIS-FJD) and Hospital Universitario Infantil Niño Jesús. Rocket has in-licensed RP-L301, a lentiviral vector (LVV)-based gene therapy for the treatment of PKD, developed by CIEMAT-CIBERER/IIS-FJD and Dr. José-Carlos Segovia, leader of the project and PKD Scientific Advisor. Rocket anticipates RP-L301 will enter the clinic during the second half of this year.

"At Rocket, we are making significant progress towards developing transformative gene therapies for patients living with devastating, rare diseases, including PKD," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Executive Vice President of Development of Rocket. "We are proud to work with our partners at CIEMAT-CIBERER/IIS-FJD and other institutions worldwide to shine a spotlight on PKD's impact on patients' lives as we advance towards a potential cure."

"We are pleased to sponsor the 3rd Annual PKD Patients' Forum to discuss PKD diagnosis and present and future treatments, including the forthcoming gene therapy clinical trial for PKD," said Dr. Segovia. "Internationally recognized PKD experts, as well as clinicians in close contact with the patients are attending today's Forum. Our objective is to educate families and patients and discuss their physical, psychological and social needs."

Dr. Segovia continued, "Patients with severe PKD suffer greatly as the only treatment options currently available to them are splenectomy and lifelong red blood cell transfusions. Unfortunately, these treatment options are far from optimal and are associated with considerable side effects. The experts at this event have devoted countless years in research and are an instrumental resource to patients and their families."

Sponsoring PKD Day is part of Rocket's commitment to promoting rare disease education and awareness. Additional recent initiatives include a Rocket-sponsored Rare Disease Day educational event on February 28 to show support for those living with rare diseases, like PKD. To celebrate this significant milestone, for the first time ever, The Empire State Building, Rocket's headquarters, shined its world-famous lights in Rare Disease Day colors. For more information about Rare Disease Day hosted by Rocket, go to https://www.rocketpharma.com/patients-and-families/rare-disease-day/

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 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.

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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