Rocket Pharmaceuticals Announces Updated Positive Preliminary Clinical Data from Phase 1 Trial of RP-L301 for the Treatment of Pyruvate Kinase Deficiency

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— Updated Data Demonstrate Sustained Improvements in Hemoglobin to Normal Range at 6-months in First Patient and Similar 3-month Improvements in Second Patient Treated —

— Maintained Safety and Tolerability of RP-L301 up to 6-months Post-treatment —

— Long-Term Data on Track for 2H2021 —

CRANBURY, N.J.--(BUSINESS WIRE)--Mar. 22, 2021-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces updated preliminary clinical data from its Phase 1 trial of RP-L301 for the treatment of Pyruvate Kinase Deficiency (PKD), showing durable normalization of hemoglobin levels up to 6 months following therapy and similar 3-month trends in the second patient treated. Additionally, results showed sustained safety and tolerability of RP-L301 6 months after treatment.

"The positive updates on our PKD trial represent continued validation of RP-L301's robust potential benefit in addressing the challenges of PKD. We are pleased to report that updated patient 1 results show that RP-L301 continues to safely normalize hemoglobin levels as demonstrated by an increase from an average of approximately 7.4 g/dL at baseline to 13.9 g/dL at 6-months post-treatment with no transfusion requirements after hematopoietic reconstitution," said Jonathan Schwartz, M.D. Chief Medical Officer and Senior Vice President of Rocket. "A similar trend in hemoglobin and bilirubin normalization were observed at three months post-treatment in patient 2. We are excited to share this data as results show the potential of RP-L301 to address the extensive burden PKD places on patients and their families. We look forward to presenting more comprehensive and updated data at a scientific venue this year."

Key findings are highlighted below. Further information about the study can be found here.

Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: A Global Phase 1 Study for Adult and Pediatric Patients

Two adult patients with significant anemia and transfusion requirements were treated with RP-L301, Rocket's *ex vivo* lentiviral gene therapy candidate for PKD. Respectively, patients L301-006-1001 and L301-001-1002 were 31- and 47-years old at the time of enrollment and have been followed for 6- and 3-months post treatment at the time of data cutoff.

- RP-L301 continued to be well tolerated, with no serious safety issues or infusion-related complications observed up to 6-months post treatment
- Patient L301-006-1001 received a cell dose of $3.9x10^6$ cells/kilogram (kg)
  - Normalized hemoglobin levels from an average baseline of ~7.4 grams (g)/deciliter (dL) to 13.9 g/dL at 6-months post treatment with RP-L301
  - Significant improvement of bilirubin, which had been substantially elevated prior to study enrollment
- Patient L301-001-1002 received a cell dose of $2.4x10^6$ cells/kg
  - Normalized hemoglobin levels from a baseline of ~7.0 g/dL to 13.8 g/dL at 3-months post treatment with RP-L301
  - Normalization of bilirubin, which had been substantially elevated prior to study enrollment

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 blood 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 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).

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of genetic therapies that correct the root cause of complex and rare childhood 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 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-K for the year ended December 31, 2020, filed March 1, 2021 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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