Rocket Pharmaceuticals Announces Preliminary Data from Phase 1/2 Trial of RP-L201 for Leukocyte Adhesion Deficiency-I

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- Initial Data Demonstrated Robust CD18 Expression and Resolution of Disease-Mediated Lesions in Months After Gene Therapy Treatment -

NEW YORK--(BUSINESS WIRE)--Dec. 9, 2019-- 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 preliminary data from its Phase 1/2 clinical trial of RP-L201, the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Leukocyte Adhesion Deficiency-I (LAD-I). LAD-I is a rare immune disorder characterized by low or absent neutrophil CD18 expression, predisposing affected individuals to recurrent and fatal infections in childhood. Data from the first patient treated with RP-L201 demonstrated early evidence of efficacy.

"We believe the preliminary data from this first patient are impressive and demonstrate that RP-L201 has the potential to correct deficient CD18 expression that is the hallmark of LAD-I," said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. "In LAD-I, stem cells are not believed to be compromised by the underlying disorder. Based on this, and an established RP-L201 treatment process that includes a tailored conditioning regimen, we intend to create a standardized and predictable platform for reversing LAD-I. We believe this treatment approach will enable us to reliably correlate drug product to both early and long-term patient outcomes. We look forward to continued evaluation of this first-in-class gene therapy in the ongoing trial and believe that RP-L201 has the potential to be a favorable therapeutic option for this difficult and frequently fatal disease."

Initial results from the first pediatric patient treated with RP-L201 demonstrate early evidence of safety and potential efficacy. Analyses of peripheral vector copy number (VCN) and CD18-expressing neutrophils were performed through three months after infusion of RP-L201 to evaluate engraftment and phenotypic correction. The patient exhibited early signs of engraftment with myeloid-lineage VCN levels of 1.5 at three months and CD18 expression of 45%, compared to pre-treatment CD18 expressions of <1%. The patient also displayed visible improvement of multiple disease-related skin lesions after receiving therapy. The drug product VCN was 3.8. No safety or tolerability issues related to RP-L201 administration (or investigational product) have been identified to-date. These data are consistent with Rocket's preclinical studies, which demonstrated that administration of RP-L201 in murine models resulted in stable engraftment and phenotypic correction with restored neutrophil migration capability.

Donald Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA and principal investigator of the trial said, "Natural history studies indicate a correlation between higher CD18 expression and survival, with 4% to 10% CD18 expression associated with survival into adulthood. Natural history studies have also demonstrated a devastating mortality rate in children with severe LAD-I, as high as 60-75% by 2 years of age, and very limited survival beyond age 10. For these reasons, we're encouraged to see preliminary evidence that investigational RP-L201 may lead to an increase in the percent of CD18-expressing neutrophils, suggesting it has potential as a life-changing, life-saving treatment option for these young patients."

The non-randomized open-label Phase 1/2 study of RP-L201 is designed to evaluate the safety and efficacy of RP-L201 in pediatric patients with severe LAD-I and is expected to enroll nine patients globally. The Phase 1 portion of the trial is expected to enroll two patients and will assess the safety, tolerability and preliminary efficacy of RP-L201. The Phase 2 portion of the trial will evaluate overall survival at several leading U.S. and EU centers. RP-L201 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 lentiviral vector was developed in a collaboration between The University College of London (UCL) and CIEMAT. Further information about the clinical program is available here.

About Leukocyte Adhesion Deficiency-I

Severe Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by mutations in the *ITGB2* gene encoding for the beta-2 integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. As a result, children with severe LAD-I (less than 2% normal expression) are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Children who survive infancy experience recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia. Without a successful bone marrow transplant, mortality in patients with severe LAD-I is 60-75% prior to the age of 2 and survival beyond the age of 5 is

uncommon. There is a high unmet medical need for patients with severe LAD-I.

Rocket's LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (Grant Number CLIN2-11480). The contents of this press release are solely the responsibility of Rocket and do not necessarily represent the official views of CIRM or any other Agency of the State of California.

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 childhood disorders. The company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients contending 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, 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 adeno-associated virus (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 pre-clinical 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, filed November 8, 2019. 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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