Rocket Pharmaceuticals Announces Clearance of IND for RP-L201 Gene Therapy for Leukocyte Adhesion Deficiency-I (LAD-I)

November 19, 2018

- Phase 1/2 Clinical Trial in Support of Registration to Commence in the U.S. in 2019 -
- Trial to Assess Safety, Tolerability and Efficacy of RP-L201 -

NEW YORK--(BUSINESS WIRE)--Nov. 19, 2018-- Rocket Pharmaceuticals, Inc. (Nasdaq:RCKT) ("Rocket"), a leading U.S.-based multi-platform 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-L201. RP-L201 is the Company's lentiviral vector (LVV)-based gene therapy for the treatment of severe Leukocyte Adhesion Deficiency-I (LAD-I) that was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT).

"This acceptance marks the second Rocket-Sponsored IND cleared for our *ex-vivo* lenti platform this quarter. We are excited to achieve this important milestone ahead of schedule, and look forward to working with the medical and patient communities to evaluate RP-L201 next year," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "This IND enables Rocket to initiate a Phase 1/2 clinical trial to assess the safety, tolerability, and efficacy of severe LAD-I in children. Severe LAD-I is a devastating pediatric disease and we believe that we may have an opportunity to significantly improve the lives of very young children suffering from this disease with a potentially curative therapy."

The planned open-label, single-arm, Phase 1 portion of the clinical trial of RP-L201 is expected to enroll two severe LAD-I patients in the U.S. The Phase 2 portion of the clinical trial of RP-L201 is expected to be a registration-enabling global study with clinical sites in the U.S., U.K. and Spain.

About Leukocyte Adhesion Deficiency-I

Severe Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, life-threatening, autosomal recessive pediatric disease caused by a mutation of the *ITGB2* gene that encodes for the Beta-2 Integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and enables neutrophil extravasation from blood vessels to combat infections. As a result, children with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial 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 exceedingly rare. The unmet medical need for patients with severe LAD-I is therefore significant.

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. Preclinical studies of additional bone marrow-derived disorders are ongoing and target 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 an undisclosed rare pediatric 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, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), 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, Rocket's ability to commence a registrational study in FA within the projected time periods, 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, 2017. 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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