Rocket Pharmaceuticals Presents Preclinical Data at the 2018 Annual Congress of the European Society of Gene and Cell Therapy (ESGCT)

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- Promising Preclinical Results from Leukocyte Adhesion Deficiency-I and Infantile Malignant Osteopetrosis Programs –

- Leukocyte Adhesion Deficiency-I Program On Track to Enter Clinic in 2019; Infantile Malignant Osteopetrosis Program in 2020-

NEW YORK--(BUSINESS WIRE)--Oct. 19, 2018-- Rocket Pharmaceuticals, Inc. (Nasdaq: RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, presents preclinical data from the Company's Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO) lentiviral vector (LVV)-based gene therapy programs at the 2018 Annual Congress of the ESGCT in Lausanne, Switzerland.

Details on Rocket's oral presentations at ESGCT:

Title: Comprehensive preclinical studies for the gene therapy of patients with leukocyte adhesion deficiency Type I (LAD-I)

Session: Blood disorders II

Presenter: Elena Almarza Novoa, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT)

RP-L201 is a LVV-based gene therapy for LAD-I that carries the ITGB2 gene that encodes the Beta-2 Integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and enables their extravasation from blood vessels to combat infections. Comprehensive safety and efficacy preclinical studies of RP-L201 have shown stable engraftment and phenotypic correction in murine models, with restored neutrophil migration capability. Additional studies tested GMP-produced RP-L201 with transduction enhancers and demonstrated increased transduction of hematopoietic progenitor cells with reproducible drug product vector copy number (VCN) in the range of 2 to 4. No safety or toxicity issues were observed.

Title: Haematopoietic stem cell targeted neonatal gene therapy by a clinically applicable lentiviral vector corrects osteopetrosis in oc/oc mice

Session: Blood disorders II

Presenter: Ilana Moscatelli, Lund University

RP-L401 is a LVV-based gene therapy for IMO that carries the TCIRG1 gene. Mutations of the TCIRG1 gene result in ineffective osteoclast function and impaired bone resorption, a process essential for normal bone growth. Bone abnormalities in IMO result in abnormal growth, debilitating neurologic abnormalities, and bone marrow failure. The disorder is often fatal during early childhood. Preclinical studies of RP-L401 were conducted in a TCIRG1-mutated oc/oc mouse model in which the defect is fatal during the initial weeks of life. RP-L401 administered shortly after birth enabled long-term survival in a majority of treated animals, restoration of bone resorption, and reversal of the osteopetrotic phenotype.

"The promising preclinical results from our LAD-I and IMO programs continue to show the potential of our LVV-based gene therapy platform to build transformative therapies for pediatric patients suffering from rare, devastating and fatal diseases. Both LAD-I and IMO represent significant areas of unmet need and are highly fatal in the early years of life without allogeneic hematopoietic stem cell transplant, which is frequently complicated by graft-versus-host disease and other significant toxicities," said Jonathan Schwartz, M.D., Chief Medical Officer of Rocket.

Dr. Schwartz continued, "Preclinical results from both programs demonstrated durable engraftment of hematopoietic stem and progenitor cells and sustained phenotypic correction of peripheral blood mononuclear cells. We are very pleased that in the LAD-I study, transduction efficiency (drug product VCN) was increased to the range of 2 to 4 with the addition of transduction enhancers. In the IMO study, there was frequent restoration of bone resorption and 75% of mice had long-term survival following administration of RP-L401. We are committed to advancing the standard of care in

these devastating hematopoietic disorders, and the continued advancement of our pipeline of five LVV and AAV-based gene therapy programs."

About Leukocyte Adhesion Deficiency-I

Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, 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 their extravasation from blood vessels to combat infections. The degree of CD18 deficiency determines the severity of the disease, which can be categorized as moderate or severe based on functional CD18 expression. Most patients have the severe form of the disease, with less than 2% of normal neutrophil CD18 expression. Severe LAD-I causes recurrent and life-threatening infections which are frequently fatal despite antibiotic use. Approximately 75% of patients die before age 2 unless an allogenic hematopoietic stem cell transplantation is performed.

About Infantile Malignant Osteopetrosis

Infantile Malignant Osteopetrosis (IMO) is a severe form of osteopetrosis most commonly caused by a genetic mutation of the TCIRG1 gene which leads to ineffective osteoclast function. Osteoclasts play a vital role in maintaining bone growth health by breaking down bone tissue through the bone resorption process and maintaining equilibrium with bone generation. Impaired bone resorption causes increased bone mass and density, skeletal deformities, debilitating neurological abnormalities and bone marrow failure. Symptoms are typically present in the first year of life and the disorder is frequently fatal within the first decade of life unless treated with an allogenic hematopoietic stem cell transplantation.

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.

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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