Rocket Pharmaceuticals Presents First Evidence of Long-Term Improvement and Stabilization in Blood Counts and Durable Mosaicism in RP-L102 “Process A” for Fanconi Anemia

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– Early Signs of Hematologic Correction in Initial Patients Who Received Adequate Drug Product–

– First Four “Process A” Patients Demonstrate Long-Term Evidence of Increasing and Durable Engraftment Resembling FA Mosaic Patients–

– Registration-Enabling Phase 2 Trial to Commence Enrollment in U.S. by Year-End –

NEW YORK--(BUSINESS WIRE)--Oct. 24, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”), a leading U.S.-based multi-platform clinical-stage gene therapy company, today presents updated long-term follow-up from the Phase 1/2 clinical trial of RP-L102 at the European Society of Cell and Gene Therapy (ESGCT) 27th Annual Congress in Barcelona, Spain. RP-L102 is the Company’s lentiviral vector (LVV)-based gene therapy for the treatment of Fanconi Anemia (FA). The data are included in an oral presentation by Dr. Juan Bueren, Scientific Director of the FA gene therapy program and Head of the Hematopoietic Innovative Therapies Division at CIEMAT in Spain / CIBERER / IIS-FJD, entitled, “Gene Therapy for Patients with Fanconi Anaemia.”

“Two critical validations for an FA gene therapy product are: 1) stem cell engraftment in the absence of cytotoxic conditioning and 2) evidence of sustained clinical improvement. We are proud to report that the maturing long-term data from the patients treated with RP-L102 meet both of these requirements,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “In all four patients, bone marrow MMC-resistance, a key measure of phenotypic reversal and engraftment, meets or exceeds the 10% threshold agreed to by the FDA and EMA for the upcoming registration-enabling Phase 2 trial, and all four patients now resemble FA mosaic patients as evaluated by peripheral T-cell chromosomal fragility assay. Remarkably, patients 02002 and 02006, who received what we consider adequate drug product similar to the upcoming Phase 2 trial, now demonstrate durable robust bone marrow MMC-resistance levels of approximately 60% and 32%, respectively, confirming phenotypic correction in long-term bone marrow stem and progenitor cells. Of note, each of the four initial patients continue to show evidence of a proliferative advantage, with ongoing increases in peripheral mononuclear cell VCNs. In addition, improvement or stabilization of peripheral blood counts, which had declined substantially prior to gene therapy, suggests a halt in bone marrow failure progression. In patient 02002, hemoglobin levels are now similar to those in the first year after birth, and all lineages in patients 02002 and 02006 are now stable or improving.”

Dr. Shah continued, “Preliminary VCN data from three additional patients who were treated with a viable drug product also show engraftment in a dose-dependent manner, consistent with the first four patients. With this progress to date, we look forward to the upcoming results from the first two patients receiving ‘Process B’ of RP-L102, designed to enable consistent results with commercial-grade product.”

The presentation described nine pediatric patients (ages 3-7 years) who received RP-L102 utilizing fresh or cryopreserved mobilized peripheral blood CD34+ cells that were transduced with the therapeutic vector. Four of these patients have been followed for more than 2 years (24-39 months for patients 02002, 02004, 02005, and 02006). The Phase 1/2 study of RP-L102 is an ongoing, open-label, single-center study designed to evaluate the safety and efficacy of “Process A” RP-L102 without the use of any conditioning regimen conventionally used in allogenic transplant.

Dr. Bueren noted, “These results indicate the feasibility of engraftment in FA patients using autologous, gene corrected HSCs in the absence of any conditioning regimen. This indicates the potential of this therapeutic approach as a definitive hematologic treatment, while avoiding the burdensome side effects associated with allogeneic transplant, including the risk of post-transplant mortality and a substantially higher risk of head and neck cancer. The ability to treat patients without the use of genotoxic conditioning and to restore blood cell counts is a life-altering advancement for patients and their families, as well as the scientific community which has dedicated over two decades to finding a minimally toxic alternative for FA patients.”

Rocket expects initial data from the Phase 1 “Process B” trial of RP-L102 by year-end. The registration-enabling Phase 2 study in Spain is now enrolling, and additional global sites will follow.

Full results from the ESGCT presentation will be available online at the conclusion of the oral presentation: https://www.rocketpharma.com/esgct-presentations/.

About Fanconi Anemia

Fanconi Anemia (FA) is a rare pediatric disease characterized by bone marrow failure, malformations and cancer predisposition. The primary cause of

death among patients with FA is bone marrow failure, which typically occurs during the first decade of life. Allogeneic hematopoietic stem cell transplantation (HSCT), when available, corrects the hematologic component of FA, but requires myeloablative conditioning. Graft-versus-host disease, a known complication of allogeneic HSCT, is associated with an increased risk of solid tumors, mainly squamous cell carcinomas of the head and neck region. Approximately 60-70% of patients with FA have a *FANC-A* gene mutation, which encodes for a protein essential for DNA repair. Mutation in the *FANC-A* gene leads to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Chromosome fragility induced by DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is the 'gold standard' test for FA diagnosis. Somatic mosaicism occurs when there is a spontaneous correction of the mutated gene that can lead to stabilization or correction of a FA patient's blood counts in the absence of any administered therapy. Somatic mosaicism, often referred to as 'nature's gene therapy' provides a strong rationale for the development of FA gene therapy because of the selective growth advantage of gene-corrected hematopoietic stem cells over FA cells[1].

[1]Soulier, J.,et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. Blood 105: 1329-1336

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 clinical programs using 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 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 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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