Rocket Pharmaceuticals Announces Clearance from the Spanish Agency for Medicines and Health Products for the Phase 2 Registration-enabling FANCOLEN-II Study of RP-L102 for Fanconi Anemia

August 22, 2019

—Phase 2 Trial to Commence Patient Enrollment in Europe—

— Rocket-sponsored Registration-enabling Study Utilizing Optimized Process B—

NEW YORK--(BUSINESS WIRE)--Aug. 22, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces clearance from the Spanish Agency for Medicines and Health Products to commence enrollment in the FANCOLEN-II Phase 2 registration-enabling study of RP-L102 for Fanconi Anemia (FA) in Spain. Hospital Infantil Universitario Niño Jesús in Madrid, in conjunction with Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto Investigación Sanitaria Fundación Jiménez Díaz (IIS-FJD), is the initial and lead center for the clinical trial under the leadership of Dr. Julián Sevilla, Clinical Investigator (M.D., Ph.D.) at the Hospital Infantil Universitario Niño Jesús and Principal Investigator for the trial.

"The Rocket and CIEMAT teams are pleased to move ahead with the late-stage development of RP-L102 for FA in Europe following a productive session with regulators and alignment on the registrational endpoints with the EMA," said 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. "After two decades of perseverance and close collaboration with the FA community, this clearance from the Spanish Authority to begin patient enrollment marks a landmark step forward toward enabling a minimally toxic hematologic cure for those who matter the most, our FA patients."

"We are pleased to have a clear path forward with this important Rocket-sponsored registration-enabling trial. Together with the ongoing Phase 1/2 FANCOLEN-I trial in Europe, the U.S. Phase 1 trial at Stanford University, and the planned U.S. Phase 2 trial, the FANCOLEN-II trial has the potential to satisfy requirements towards the overall global registration package for RP-L102," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Across these studies, we are using commercial grade 'Process B', which will enable consistency and improved efficiencies across studies for global licensure."

The European Medicines Agency (EMA) agreed to several key elements of the open-label Phase 2 trial, including endpoints that are designed to demonstrate reversion of the sensitivity of hematopoietic cells to DNA-damaging agents. The primary endpoint of the trial is the emergence of mitomycin-C (MMC) resistance in bone marrow colony forming (progenitor) cells. A surrogate endpoint is a diepoxybutane (DEB) chromosomal stability of peripheral blood T-lymphocytes. Sensitivity to DNA-damaging agents (MMC and DEB) is a phenotypic hallmark of FA. Additional outcomes include stability or increase in blood counts with no significant worsening in anemia, neutropenia or thrombocytopenia and peripheral blood genetic correction, as demonstrated by vector copy number (VCN), including progressive increases in peripheral blood VCN over the months subsequent to infusion.

The open-label Phase 2 clinical trial will enroll up to five pediatric patients in Europe. The study will enroll younger patients who have not developed severe bone marrow failure. The patients will receive a single intravenous infusion of RP-L102 that utilizes "Process B" which incorporates enhanced stem cell enrichment, transduction enhancers and commercial-grade vector and manufacturing without the use of any conditioning treatment. The study is designed to assess the benefit/risk profile of RP-L102.

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, which is highly toxic for the patient. HSCT is frequently complicated by graft versus host disease and also increases the risk of solid tumors, particularly upper aerodigestive tract squamous cell carcinomas. Approximately 60-70% of patients with FA have a *FANCA* gene mutation, which encodes for a protein essential for DNA repair. Mutations in the *FANCA* 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. These assays can further differentiate FA patients from mosaic patients. Somatic mosaicism occurs when there is a spontaneous reversion mutation that can lead to a mixed chimerism of corrected and uncorrected bone marrow cells leading to stabilization or correction of an FA patient's

blood counts in the absence of any administered therapy. Somatic mosaicism provides strong rationale for the development of FA gene therapy and demonstrates the selective advantage of gene-corrected hematopoietic cells in FA[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 first two 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, and Leukocyte Adhesion Deficiency-I (LAD-I), a serve pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal. 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 programs for bone marrow-derived disorders are for Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO). 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190822005179/en/

Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP, Strategy & Corporate Development
Rocket Pharma, Inc.
The Empire State Building, Suite 7530
New York, NY 10118
www.rocketpharma.com
investors@rocketpharma.com