Rocket Pharmaceuticals Announces Registration-Enabling Phase 2 Plans for RP-L102 Gene Therapy for Fanconi Anemia Following a Supportive End-of-Phase 1 FDA Meeting

September 4, 2019

— U.S. Phase 2 Trial to Use MMC-Resistance as Primary Endpoint, Supported by Additional Blood Count, Genetic and Phenotypic Assessments —

— Global Product Licensure Strategy Aligned Across U.S. and E.U.—

NEW YORK--(BUSINESS WIRE)--Sep. 4, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces the U.S. Phase 2 clinical development plan for RP-L102, the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Fanconi Anemia (FA). Based on feedback from a recent End-of-Phase 1 meeting with the U.S. Food and Drug Administration (FDA), Rocket plans to open enrollment for the U.S. Phase 2 trial of RP-L102 for FA in the fourth quarter of 2019.

"Rocket's End-of-Phase 1 meeting with the FDA was an important milestone for advancing the development of RP-L102 for FA," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We are thankful for the Agency's collaboration and guidance on the Phase 2 study design which will enable an efficient registration path to potentially bring our first-in-class gene therapy to market for FA patients in need. One key aspect of the agreed upon design is the primary endpoint of resistance to mitomycin-C, a DNA damaging agent, in bone marrow stem cells at a minimum time point of one year. Depending on the totality of available clinical evidence at the time of filing, MMC-resistance may also serve as a surrogate endpoint for an accelerated approval. These developments have enabled us to advance the development of our FA program more efficiently than anticipated."

"The acute and long-term side effects associated with allogeneic stem cell transplantation, the current standard of care for FA, emphasize the urgent need for additional curative hematologic strategies in this devastating pediatric disease," said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. "In the Spanish Phase 1 trial, RP-L102 has shown strong safety and promising efficacy to date, with durable engraftment in peripheral blood and bone marrow. Moreover, in patients followed long-term, we have now seen the emergence of blood and marrow profiles resembling those of FA patients in whom a population of blood forming stem cells spontaneously reverted to a normal phenotype. We believe RP-L102 can serve as a low-toxicity treatment without chemotherapy conditioning early in the patient's disease course aimed at preventing progression to bone marrow failure. In contrast to allogeneic stem cell transplant, this approach has no risk of graft-versus-host disease, a complication that not only increases risk of death from infection but also the risk of head and neck cancer—a major cause of mortality in FA."

Based on the combined feedback from the FDA and the Europe Medicines Agency (EMA), the pivotal study data in support of FA product licensure application is anticipated to be based on the combined U.S. and E.U. Phase 2 studies. The Center for Definitive and Curative Medicine at Stanford University and Hospital Infantil Universitario Niño Jesús will serve as the lead clinical sites in the U.S. and E.U., respectively. Additional clinical sites may be added in the global Phase 2 studies. The proposed overall safety database is anticipated to have a total exposure of 21 patients treated in the Phase 1/2 FANCOLEN-I trial in Europe (n=9), the U.S. Phase 1 trial at Stanford University (n=2), and the planned U.S. and European (FANCOLEN-II) Phase 2 trials (n=5 per region). The planned Phase 2 clinical trial will enroll up to five patients in the U.S. with no upper age limit and who have not developed severe bone marrow failure. The patients will receive a single intravenous infusion of RP-L102 that utilizes fresh cells and "Process B" which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product. "Process B" is currently being utilized in the ongoing U.S. Phase 1 trial of RP-L102 and the Phase 2 FANCOLEN-II trial in Europe.

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 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/20190904005325/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