Rocket Pharmaceuticals Receives the European Medicines Agency PRIME Eligibility for RP-L102 Gene Therapy for Fanconi Anemia

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- Milestone Gives Fanconi Anemia Program All Accelerated Regulatory Tools in U.S. and EU, Including FDA Regenerative Medicine Advanced Therapy, Fast Track and Orphan Designations -

NEW YORK--(BUSINESS WIRE)--Dec. 16, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces that the European Medicines Agency (EMA) has granted PRIority MEdicines (PRIME) eligibility to RP-L102, the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Fanconi Anemia (FA). PRIME is a program launched by the EMA to enhance support for the development of medicines that target an unmet medical need. With this milestone, Rocket's FA program has received all accelerated regulatory designations in the U.S. and EU, including FDA Regenerative Medicine Advanced Therapy, Fast Track and Orphan designations.

PRIME eligibility was granted based on long-term data from patients treated with RP-L102 "Process A." These data demonstrate that treatment with RP-L102 results in increasing and durable engraftment, with patients approaching a FA mosaic phenotype. These findings suggest that RP-L102 leads to phenotypic reversal and sustained clinical improvement.

"We are pleased to receive PRIME eligibility for RP-L102. This PRIME designation, qualified by the positive data reported from FA 'Process A,' further confirms that patients living with FA are severely underserved by current treatment options," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development of Rocket. "This validates the urgent need for new treatments for young children affected by Fanconi Anemia and allows us the opportunity to collaborate closely with the EMA to accelerate the clinical development of RP-L102 in the European Union. The collaborative involvement of the EMA and FDA will help enable expeditious development of FA gene therapy over the coming year."

The PRIME program aims to optimize development plans and speed up evaluation of medicines that may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options. These medicines are considered priority medicines by the EMA and are intended to reach patients earlier. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.

Rocket's global registrational Phase 2 trial of RP-L102 is currently ongoing. Patients will be treated with RP-L102 "Process B" which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product. The primary endpoint, which has been agreed upon by both the EMA and U.S. Food and Drug Administration (FDA), will be the emergence of MMC-resistance, a measure of phenotypic correction, in bone marrow cells.

Hospital Infantil Universitario Niño Jesús and Lucile Packard Children's Hospital Stanford are serving as the lead clinical sites and University of Minnesota is conducting centralized evaluation of bone marrow MMC-resistance and engaging in advisory activities for the global trial of RP-L102. RP-L102 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER), Instituto de Investigación Sanitaria Fundación Jiménez Díaz (IIS-FJD) and Fundacion para la Investigacion Biomedica Hospital Infantil Universitario Niño Jesus (FIB-HIUNJ).

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 'natural 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 advancing an integrated and sustainable pipeline of genetic therapies that correct the root cause of complex and rare childhood disorders. The company’s platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients contending with rare genetic diseases. 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 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, filed November 8, 2019. 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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