Rocket Pharmaceuticals Announces European Medicines Agency Acceptance of RP-L102 Marketing Authorization Application for the Treatment of Fanconi Anemia

April 2, 2024

Positive, previously disclosed results from the global Phase 1/2 trial demonstrated genetic and phenotypic correction combined with hematologic stabilization extending out to 42 months after treatment with RP-L102

CRANBURY, N.J.--(BUSINESS WIRE)--Apr. 2, 2024-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced that the European Medicines Agency (EMA) accepted the Marketing Authorization Application (MAA) for RP-L102, its lentiviral (LV) vector-based investigational gene therapy for Fanconi Anemia (FA), complementation group A, a rare genetic disorder caused by mutations in the *FANCA* gene affecting DNA repair and characterized by bone marrow failure (BMF), cancer predisposition, and congenital malformations.

"The acceptance of the MAA for RP-L102 marks an important step forward in our goal of bringing this potential gene therapy treatment to patients impacted by this devastating childhood disorder. Currently, there are no existing options to potentially prevent BMF for patients with FA," said Kinnari Patel, Pharm.D., MBA, President, Head of R&D and Chief Operating Officer, Rocket Pharma. "We are appreciative of the patients, their families, and researchers who helped reach this meaningful milestone and continue participating in the clinical development program. We look forward to partnering closely with the EMA throughout the review process to make RP-L102 available to patients with FA who are in need of new treatment options."

MAA acceptance was based on positive, previously disclosed data from the global RP-L102 Phase 1/2 clinical trial. RP-L102 demonstrated sustained genetic correction, comprehensive phenotypic correction, and hematologic stabilization. The safety profile was highly favorable with no significant safety signals, and the treatment, administered without any cytotoxic conditioning, was well tolerated. There were no signs of bone marrow dysplasia, clonal dominance, or insertional mutagenesis related to RP-L102.

In the absence of allogeneic hematopoietic stem cell transplant (HSCT), the primary cause of death among patients with FA is BMF, which typically occurs during the first decade of life. Although allogeneic transplants can cure the hematologic component of FA, they confer significant side effects and substantially increase the risk of solid organ malignancies, which have become the most frequent cause of FA-related death.

The Biologics License Application (BLA) for FA remains on track for submission to the U.S. Food and Drug Administration (FDA) in the first half of 2024.

About RP-L102

RP-L102 is an investigational gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral (LV) vector to contain a functional copy of the *FANCA* gene. Rocket holds FDA Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric Disease, and Fast Track designations in the U.S., PRIME and Advanced Therapy Medicinal Product (ATMP) designations in the EU, and Orphan Drug designation in both regions for the program. RP-L102 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras and Instituto de Investigación Sanitaria Fundación Jiménez Díaz.

About Fanconi Anemia

Fanconi Anemia (FA) is a rare genetic disorder characterized by bone marrow failure (BMF), cancer predisposition, and congenital malformations. In the absence of allogeneic hematopoietic stem cell transplant (HSCT), the primary cause of death among patients with FA is BMF, which typically occurs during the first decade of life. Allogeneic HSCT, when available, corrects the hematologic component of FA, but requires myeloablative conditioning. Both chemotherapy conditioning and graft-versus-host disease, a known complication of allogeneic HSCT, are 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 Fanconi Anemia complementation group A (*FANCA*) gene mutation, which encodes for a protein essential for DNA repair. Mutations in the *FANCA* gene lead to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Increased sensitivity to DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is a "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. There is a high unmet medical need for

patients with FA.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. Rocket's innovative multi-platform approach allows us to design the optimal gene therapy for each indication, creating potentially transformative options that enable people living with devastating rare diseases to experience long and full lives.

Rocket's lentiviral (LV) vector-based hematology portfolio consists of late-stage programs for Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure (BMF) 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 monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

Our adeno-associated viral (AAV) vector-based cardiovascular portfolio includes a late-stage program for Danon Disease, a devastating heart failure condition resulting in thickening of the heart, an early-stage program in clinical trials for PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death, and a pre-clinical program targeting BAG3-associated dilated cardiomyopathy (DCM), a heart failure condition that causes enlarged ventricles.

For more information about Rocket, please visit www.rocketpharma.com and follow us on LinkedIn, YouTube, and X.

Rocket Cautionary Statement Regarding Forward-Looking Statements

This press release contains forward-looking statements concerning Rocket's future expectations, plans and prospects that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this release are forward-looking statements. 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. These forward-looking statements include, but are not limited to, statements concerning Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its DD program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, Rocket's ability to establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates and Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies. 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 dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket's competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket's ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, Rocket's ability to acquire additional businesses, form strategic alliances or create joint ventures and its ability to realize the benefit of such acquisitions, alliances or joint ventures, Rocket's ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, 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, 2023, filed February 27, 2024 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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/20240402101364/en/

Media
Kevin Giordano
media@rocketpharma.com

Investors
Brooks Rahmer
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.