# Rocket Pharmaceuticals Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for RP-L301 Gene Therapy for Pyruvate Kinase Deficiency (PKD)

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CRANBURY, N.J.--(BUSINESS WIRE)--May 23, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-L301, the Company's investigational lentiviral-based gene therapy for Pyruvate Kinase Deficiency (PKD), a rare blood disorder characterized by severe anemia and excessive red blood cell breakdown. RMAT designation was granted based on robust safety and efficacy data from the ongoing Phase 1 RP-L301 clinical trial and its potential to cure a life-threatening disease for which no curative therapies currently exist. The designation will provide the benefits of added FDA guidance and expedited review through the program's development.

"Receiving RMAT designation from the FDA for RP-L301 is a major achievement in our pursuit to bring the first, potentially curative gene therapy treatment to patients living with PKD who have high unmet need. Notably, PKD has an estimated prevalence of up to 8,000 patients in the U.S. and Europe and represents one of the most significant patient opportunities in our LV hematology portfolio," said Kinnari Patel, PharmD, MBA, President and Chief Operating Officer, Rocket Pharma. "Further, all four Rocket-sponsored programs with clinical data now have received RMAT designation from the FDA across both platforms, a unique showcase of our team's ability both to select appropriate targets and develop gene therapies for them."

Dr. Patel continued, "Results from the RP-L301 program demonstrate robust efficacy in both adult patients for up to 30 months with a highly favorable safety profile and were recently presented at ASGCT. The first pediatric patient has shown promising initial results similar to the adults, and enrollment has been completed in the Phase 1 study. We look forward to initiating the Phase 2 pivotal trial in the fourth quarter of 2023 as we continue to advance our world-class pipeline for patients facing such rare and devastating diseases."

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising pipeline products, including gene therapies. A regenerative medicine therapy is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or therapy has the potential to address unmet medical needs for such disease or condition.

Similar to Breakthrough Therapy designation, RMAT designation provides the benefits of intensive FDA guidance on efficient drug development, including the ability for early FDA interactions to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the biologics license application (BLA) and other opportunities to expedite development and review. In addition to RMAT, RP-L301 has also received Fast Track and Orphan Drug Designation.

Results from the Phase 1 program presented recently at the 26$^{th}$ Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) demonstrate robust and sustained efficacy in both adult patients up to 30 months post-infusion demonstrated by normalized hemoglobin (from baseline levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, red blood cell transfusion independence and improved quality of life with documented improvements via formal quality of life assessments. The safety profile appears highly favorable, with no RP-L301-related serious adverse events in either of the adult patients.

The first pediatric patient results suggest efficacy similar to the adult cohort with an initial greater than five-point increase in hemoglobin (from median baseline level of 7.9 g/dL). The infusion was well tolerated, with engraftment achieved at day +15, hospital discharge less than one month following infusion, and no RP-L301-related serious adverse events or red blood cell transfusion requirements following engraftment.

**About Pyruvate Kinase Deficiency**

Pyruvate Kinase Deficiency (PKD) is a rare, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red blood cell destruction and the disorder ranges from mild to life-threatening anemia. PKD has an estimated prevalence of 4,000 to 8,000 patients in the U.S. and Europe. Children are the most commonly and severely affected subgroup of patients. Patients with PKD have a high unmet medical need, as currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload. Recently, mitapivat, an oral enzyme activator, was approved for use in adult patients, however its efficacy is limited in more severely-afflicted patients, most notably in those who are splenectomized, transfusion-dependent, or whose disease results from deleterious mutations.

RP-L301 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz (IIS-FJD).

**About Rocket Pharmaceuticals, Inc.**

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (LV) 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 adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. Rocket also has preclinical AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and BAG3-associated dilated cardiomyopathy (DCM). 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 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 Danon Disease program, including its planned pivotal trial, 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 "aim," "anticipate," "believe," "can," "continue," "design," "estimate," "expect," "intend," "may," "plan," "potential," "will give," "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 monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any 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, 2022, filed February 28, 2023 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.

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