Rocket Pharmaceuticals Announces FDA Acceptance of Biologics License Application with Priority Review for RP-L201 (marnetegragene autotemcel) for the Treatment of Severe Leukocyte Adhesion Deficiency-I (LAD-I)

October 2, 2023

Prescription Drug User Fee Act (PDUFA) target action date is March 31, 2024

RP-L201 has received FDA Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric, Fast Track and Orphan Drug designations; Rocket eligible for Priority Review Voucher, if RP-L201 is approved

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 2, 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 accepted the Biologics License Application (BLA) and granted Priority Review for RP-L201 (marnetegragene autotemcel), a lentiviral vector (LV)-based investigational gene therapy for severe Leukocyte Adhesion Deficiency-I (LAD-I), a rare genetic immune disorder that predisposes patients to recurrent and fatal infections and is near-uniformly fatal in childhood without an allogeneic hematopoietic stem cell transplant. The PDUFA date set by the FDA is March 31, 2024.

“Today’s acceptance of the BLA by the FDA marks a significant milestone for Rocket towards our goal of delivering a one-time gene therapy to patients facing the devastating effects of severe LAD-I. For these patients, survival beyond childhood is uncommon. Bone marrow transplant is currently the only treatment option, has substantial morbidity and mortality, and may not be available in time for these children,” said Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer, Rocket Pharma. “We are incredibly grateful to the patients, caregivers and researchers who have shared this journey with us and look forward to continuing our close collaboration with the FDA during the review period as we work to bring RP-L201 to patients as quickly as possible.”

Positive top-line data from the global Phase 1/2 study of RP-L201 demonstrated 100% overall survival at 12 months post-infusion (and for the entire duration of follow-up) for all nine LAD-I patients with 12 to 24 months of available follow-up. Data also showed large decreases compared with pre-treatment history in the incidences of significant infections, combined with evidence of resolution of LAD-I-related skin lesions and restoration of wound repair capabilities. All primary and secondary endpoints were met, and RP-L201 was very well tolerated in all patients with no treatment related SAEs.

“As the Principal Investigator in the U.S., I oversaw treatment of six of the nine LAD-I patients in this trial. In my opinion, the results are remarkable. All of these children have been in good health with no significant LAD-I-related infections or inflammatory skin lesions since treatment. Based on what I see, they are all experiencing a normal childhood life, which is the goal of this type of potentially curative gene therapy,” said Donald B. Kohn, M.D., Distinguished Professor of Microbiology, Immunology & Molecular Genetics, Pediatrics, and Molecular & Medical Pharmacology at University of California, Los Angeles (UCLA) and Director of the UCLA Human Gene and Cell Therapy Program.

About RP-L201 (marnetegragene autotemcel)

RP-L201 is an investigational gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral vector to deliver a functional copy of the *ITGB2* gene, which encodes for the beta-2 integrin component CD18, a key protein that facilitates leukocyte adhesion and enables their extravasation from blood vessels to fight infection. Rocket holds FDA Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric, 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-L201 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. The lentiviral vector was developed in a collaboration between UCL and CIEMAT.

About Leukocyte Adhesion Deficiency-I

Severe Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by mutations in the *ITGB2* gene encoding for the beta-2 integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. As a result, children with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Children who survive infancy experience

recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia. Without a successful bone marrow transplant, survival beyond childhood is rare. LAD-I is estimated to impact an estimated 800 to 1,000 individuals in the U.S. and Europe. Currently the only potential curative treatment is an allogeneic hematopoietic stem cell transplant, which may not be available in time for these children and itself has substantial morbidity and mortality. There is a high unmet medical need for patients with severe LAD-I.

Rocket's LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (Grant Number CLIN2-11480). The contents of this press release are solely the responsibility of Rocket and do not necessarily represent the official views of CIRM or any other agency of the State of California.

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 is developing 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 "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 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, our expectations regarding the delays and impact of COVID-19 on clinical sites, 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, 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, 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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