Rocket Pharmaceuticals Announces FDA Acceptance of BLA Resubmission of KRESLADI™ for the Treatment of Severe Leukocyte Adhesion Deficiency-I (LAD-I)

October 14, 2025

Prescription Drug User Fee Act (PDUFA) target action date is March 28, 2026

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Rocket is eligible for a Rare Pediatric Disease Priority Review Voucher, should KRESLADI ™be approved

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 14, 2025-- 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 U.S. Food and Drug Administration (FDA) has accepted the resubmission of the Biologics License Application (BLA) for KRESLADI™ (marnetegragene autotemcel; marne-cel), 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 28, 2026.

"We value the continued dialogue with the FDA and believe the BLA moves Rocket closer to 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, mortality, and cost, and may not be available in time for these children," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "As we approach our new PDUFA date, we are focused on the opportunity to make this therapy available to patients who need it most."

The BLA is supported by positive clinical efficacy and safety data from the global Phase 1/2 study of KRESLADI™, which demonstrated 100% overall survival at 12 months post-infusion (and for the entire duration of follow-up) for all enrolled patients. All primary and secondary endpoints were met, and KRESLADI™ was well tolerated in all patients with no treatment-related serious adverse events. The data showed substantial reductions in the incidence of significant infections compared to pre-treatment levels, along with evidence of improvement in skin lesions and restoration of wound-healing capabilities.

Rocket is eligible for a Rare Pediatric Disease Priority Review Voucher (PRV), should KRESLADI™ be approved.

About KRESLADI™ (marnetegragene autotemcel; marne-cel)

KRESLADI™ is an investigational gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral (LV) 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 designations in both regions for the program. KRESLADI™ 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 University College London and CIEMAT.

About Leukocyte Adhesion Deficiency-I

Leukocyte Adhesion Deficiency-I (LAD-I) is an ultra-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 to the blood vessel wall and migration to tissues to confine and clear infections and orchestrate wound repair. As a result, children with severe LAD-I have high unmet need and are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antimicrobials and require frequent hospitalizations. Approximately 61-69% of children with LAD-I are classified as severe cases of LAD-I. Survival beyond childhood is uncommon, with approximately 60-75% of patients dying before age two in the absence of a transplant procedure. Children with severe LAD-I who survive infancy experience recurrent life-threatening bacterial and fungal infections; intractable, nonhealing skin lesions; and chronic inflammation including severe periodontitis.

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

Rocket's lentiviral (LV) vector-based hematology portfolio consists of late-stage programs for Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure (BMF) and potentially cancer, and Pyruvate Kinase Deficiency (PKD), a monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

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 "could," "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 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, including the timing and outcome of the FDA's review of the additional CMC information that Rocket will provide in response to the FDA's request, the safety, effectiveness and timing of 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, Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies, Rocket's ability to transition to a commercial stage pharmaceutical company, and Rocket's expectation that its cash, cash equivalents and investments will be sufficient to fund its operations into the second quarter of 2027. 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, the integration of new executive team members and the effectiveness of the newly configured corporate leadership team, 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, 2024, filed February 27, 2025 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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