Rocket Pharmaceuticals Announces FDA Approval of KRESLADI™ for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)

March 27, 2026

First FDA-approved gene therapy for children with severe LAD-I due to biallelic variants in *ITGB2*

Severe LAD-I is an ultra-rare, life-threatening pediatric genetic immunodeficiency characterized by recurrent infections and high early-childhood mortality without treatment

FDA grants Rare Pediatric Disease Priority Review Voucher

Company to host conference call today, March 27 at 8:30 AM ET

CRANBURY, N.J.--(BUSINESS WIRE)--Mar. 27, 2026-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated 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 granted accelerated approval for KRESLADI™ (marnetegragene autotemcel), an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen-matched sibling donor for allogeneic hematopoietic stem cell transplant. This indication is approved under accelerated approval based on increase in neutrophil CD18 and CD11a surface expression. Confirmation of clinical benefit will be based on the evaluation of longer-term follow-up data of treated patients in the ongoing clinical study and through a post-marketing registry.

With the approval of KRESLADI, the FDA granted Rocket a Rare Pediatric Disease Priority Review Voucher (PRV), a program designed to encourage development of therapies for rare pediatric diseases. The Company intends to evaluate strategic options to monetize the PRV in a manner designed to enhance financial flexibility and maximize shareholder value.

“The approval of KRESLADI represents an important milestone for the severe LAD-I community,” said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharmaceuticals. “This approval reflects the dedication of patients, families, investigators, and regulators who have worked together to advance research of this ultra-rare disease. We look forward to making KRESLADI available to eligible patients in the United States.”

LAD-I is an ultra-rare genetic pediatric disease caused by mutations in the *ITGB2* gene encoding for CD18, a key protein that is expressed along CD11 integrins to facilitate leukocyte adhesion to the blood vessel wall and migration to tissues to confine and clear infections and orchestrate wound repair. Patients with severe LAD-I typically show very diminished CD11a expression. Infants with severe LAD-I suffer from recurrent, life-threatening bacterial, and fungal infections that respond poorly to antimicrobials and require frequent hospitalizations. In the U.S., the incidence of LAD-I is estimated to range from approximately one in 100,000 to one in 200,000 live births, with roughly two-thirds of affected patients classified as having the severe form of the disease.

“As a clinician, I have seen firsthand the serious impact that severe LAD-I can have on young children and their families,” said Donald B. Kohn, M.D., Principal Investigator of the Phase 1/2 study and Distinguished Professor of Microbiology, Immunology & Molecular Genetics at the University of California, Los Angeles (UCLA). “The approval of KRESLADI represents the culmination of many years of scientific research and clinical collaboration aimed at addressing the underlying cause of this devastating disease.”

“The approval of KRESLADI represents a significant development for individuals affected by severe LAD-I and the broader primary immunodeficiency community,” said Vanessa Tenembaum, Chief Executive Officer of the Jeffrey Modell Foundation, a global nonprofit organization dedicated to early diagnosis and treatments for primary immunodeficiency. “For families impacted by this rare and serious disease this approval underscores the importance of continued efforts to improve outcomes for patients with primary immunodeficiencies.”

More information is available for patients, families, and healthcare providers in the U.S. at www.KRESLADI.com, including full Prescribing Information.

Research supporting the development of KRESLADI was made possible in part by funding from the California Institute for Regenerative Medicine (Grant Number CLIN2-11480).

Conference Call Information
Rocket will host an investor conference call on March 27, 2026, at 8:30 AM ET. The conference call will be accessible at

www.webcaster5.com/Webcast/Page/3046/53682.

For those unable to listen to the live conference call, a replay will be available for 30 days on the Investors section of the Company's website at https://ir.rocketpharma.com/.

INDICATION AND USAGE
KRESLADI is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen (HLA)-matched sibling donor for allogeneic hematopoietic stem cell transplant.

This indication is approved under accelerated approval based on increase in neutrophil CD18 and CD11a surface expression. Continued approval may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Serious Infections
Serious infections have occurred with KRESLADI administration. Increased susceptibility to infections may occur due to administration of myeloablative conditioning prior to KRESLADI infusion.

Monitor patients for signs and symptoms of infection before and after KRESLADI infusion and treat appropriately. Administer prophylactic antimicrobials according to institutional guidelines.

Avoid administration of KRESLADI in patients with active bloodstream infections or other serious, untreated infections.

Any blood products required after KRESLADI infusion should be irradiated.

Veno-Occlusive Disease
Veno-occlusive disease has occurred with KRESLADI treatment. Increased susceptibility to veno-occlusive disease may occur due to administration of myeloablative conditioning prior to KRESLADI infusion. Monitor patients for signs and symptoms of veno-occlusive disease including assessment of liver function tests during the first month following KRESLADI infusion.

Neutrophil Engraftment Failure
Neutrophil engraftment failure may occur after treatment with KRESLADI. Neutrophil engraftment failure is defined as failure to achieve three consecutive absolute neutrophil counts (ANC) ≥ 500 cells/microliter obtained on different days by Day 43 after infusion of KRESLADI. Monitor neutrophil counts until engraftment has been achieved. If neutrophil engraftment failure occurs in a patient treated with KRESLADI, provide rescue treatment with the back-up collection of CD34+ cells.

Delayed Platelet Engraftment
Delayed platelet engraftment may occur after treatment with KRESLADI. Monitor platelet counts and bleeding until platelet engraftment and platelet recovery are achieved.

LVV-Mediated Insertional Oncogenesis
Lentiviral vector (LVV)-mediated insertional oncogenesis may occur after treatment with KRESLADI. Hematologic malignancy is a lifelong risk and patients treated with KRESLADI may develop hematologic malignancy at any time following treatment.

Monitor for hematologic malignancies clinically, and with a complete blood count (with differential) at least annually and integration site analysis as warranted for at least 15 years after treatment with KRESLADI and as clinically indicated. If malignancy is detected in any patient who received KRESLADI, contact Rocket Pharmaceuticals, Inc. at 1-800-982-2410 for reporting and to obtain instructions on collection of samples for testing.

Hypersensitivity Reactions
Hypersensitivity reactions including anaphylaxis may occur with the infusion of KRESLADI. The dimethyl sulfoxide (DMSO) in KRESLADI may cause hypersensitivity reactions which may occur in patients with and without prior exposure to DMSO.

Monitor patients for signs and symptoms of hypersensitivity reactions during and after KRESLADI infusion. If a hypersensitivity reaction occurs, pause infusion if ongoing and manage according to clinical practice.

Anti-Retroviral Use
Anti-retroviral medications may interfere with manufacturing of KRESLADI. If a patient requires anti-retrovirals for HIV prophylaxis, mobilization and apheresis of CD34+ cells for KRESLADI manufacturing should be delayed until HIV infection is adequately ruled out. Patients should not take anti-retroviral medications for at least one month prior to mobilization, or for the expected duration required for the elimination of the anti-retroviral medications, and until all cycles of apheresis are completed.

Interference with Serology Testing
Patients who have received KRESLADI are likely to test positive by polymerase chain reaction (PCR) assays for HIV due to LVV provirus insertion resulting in a false-positive test for HIV. Therefore, patients who have received KRESLADI should not be screened for HIV infection using a PCR-based assay.

Blood, Organ, Tissue, and Cell Donation
Patients treated with KRESLADI should not donate blood, organs, tissues, or cells for transplantation at any time in the future.

ADVERSE REACTIONS
The most common non-laboratory adverse reactions (≥ 30% of patients) include: mucositis, upper respiratory tract infection, viral infection, febrile neutropenia, skin lesion, nausea/vomiting, rash/dermatitis, pyrexia, device related infection, and skin infection.

The most common laboratory adverse reactions (≥ 30% of patients) include: hemoglobin decreased, platelet count decreased, neutrophil count

decreased, leukocyte count decreased, aspartate aminotransferase increased, and alanine aminotransferase increased.

For additional safety information, refer to the full Prescribing Information.

DRUG INTERACTIONS
No formal drug interaction studies have been performed. KRESLADI is not expected to interact with the hepatic cytochrome P-450 family of enzymes or drug transporters.

Vaccines
The safety and effectiveness of immunization with live viral vaccines during or following KRESLADI treatment has not been studied. Vaccination is not recommended during the 6 weeks preceding the start of myeloablative conditioning, and until hematological recovery following treatment with KRESLADI. Where feasible, administer childhood vaccinations prior to myeloablative conditioning for KRESLADI.

Anti-retroviral Medications
Patients should not take anti-retroviral medications for at least one month prior to initiating medications for stem cell mobilization and for the expected duration for elimination of the medications, and until all cycles of apheresis are completed. Anti-retroviral medications may interfere with manufacturing of KRESLADI.

REFERENCE TO FULL PRESCRIBING INFORMATION
Please see full Prescribing Information for KRESLADI.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated biotechnology company advancing gene therapies for rare and devastating cardiovascular diseases, with additional programs in hematology and immunology. Rocket's cardiovascular pipeline includes three clinical stage programs that each target one of the major inherited cardiomyopathy subtypes: hypertrophic, arrhythmogenic, and dilated cardiomyopathies. Together these conditions represent more than 100,000 patients in the U.S. and EU. The Company's platform is supported by proprietary AAV manufacturing capabilities, multi-year efficacy and safety data in cardiac gene therapy, and experience treating several cardiac patients across late-stage AAV programs. For more information, 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," "look forward," "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 expected commercial availability and launch timing of KRESLADI™; anticipated patient enrollment and treatment timelines; market adoption and patient identification; Rocket's commercial strategy and launch readiness; Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates; the anticipated timing and impact of commercialization activities; Rocket's projected cash runway; potential monetization of the Rare Pediatric Disease Priority Review Voucher; and the continued development and advancement of Rocket's pipeline programs. 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, risks related to: the successful launch and commercialization of KRESLADI™; the safety and efficacy profile of KRESLADI™; the Company's ability to obtain and maintain regulatory approvals; the ability to confirm clinical benefit required for continued approval under the accelerated approval pathway; manufacturing and supply chain considerations, including reliance on third-party manufacturers; the availability and readiness of qualified treatment centers; payer coverage and reimbursement; patient identification and referral dynamics; 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 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, 2025, filed February 26, 2026 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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