Pharmacy Focus:
Kresladi™ for Leukocyte Adhesion Deficiency Type 1

Key Takeaways[1-3]

Leukocyte Adhesion Deficiency type 1 (LAD-1) is a rare, life-threatening, primary immunodeficiency. Patients – most often children – experience recurrent, severe bacterial and fungal infections due to the inability of their white blood cells to fight infections.

Kresladi is the first gene therapy FDA-approved for pediatric patients with severe LAD-1 with specific mutations who do not have an available sibling donor for an allogeneic hematopoietic stem cell transplant (ALLO HSCT).

Rocket Pharmaceuticals, Inc. expects to launch Kresladi by the end of 2026. Pricing is currently unknown, but it is expected to be in the millions for the drug only, which is consistent with other gene therapies on the market.

Disease Overview[1-6]

Description: LAD-1 is an ultra-rare, inherited immune disorder caused by mutations in the ITGB2 gene. The ITGB2 gene is responsible for the production of a protein (CD18) that plays a vital role in the immune system. With reduced CD18 due to the mutation, white blood cells (leukocytes) are unable to adhere to blood vessel walls and migrate to sites of infection, leaving affected patients susceptible to life-threatening bacterial and fungal infections.

Severity: LAD-1 is generally divided into the following severity categories based on CD18 expression levels:

- Severe: CD18 expression is <2% of normal levels
- Moderate: CD18 expression is 2% to 30% of normal levels
- Mild: CD18 expression is >30% of normal levels

Occurrence: Severe LAD-1 is an ultra-rare disease with an estimated incidence of 1 in 100,000 to 1 in 200,000 live births in the United States. Approximately two-thirds of all LAD-1 cases are classified as severe.

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Treatment

The standard of care for LAD-1 has been allogeneic hematopoietic stem cell transplantation (ALLO HSCT). However, this procedure carries significant risks, including graft-versus-host disease. It also requires a well-matched donor, which often is unavailable. For these patients, Kresladi offers a form of stem cell transplantation that involves a patient's own cells being genetically modified in a lab and then infused back into the patient.

Kresladi™ [1,2,7]	
Generic Name	Marnetegragene autotemcel
Manufacturer	Rocket Pharmaceuticals, Inc.
Approval	March 26, 2026
Use	Treatment of pediatric patients with severe leukocyte adhesion deficiency-1 (LAD-1) due to biallelic variants in ITGB2 without an available HLA-matched sibling donor for ALLO HSCT
How It Works	Kresladi is an autologous hematopoietic stem cell (HSC) gene therapy; a patient's own HSCs are collected and genetically modified using a lentiviral vector to deliver a functional copy of the ITGB2 gene. The modified cells are then infused back into the patient to restore CD18 protein expression on white blood cells, allowing them to fight infections.
Procedure Location	Inpatient Hospital or Specialized Treatment Center
Timeframe	One-time infusion
Efficacy	Kresladi was studied in nine total pediatric patients with severe LAD-1 who ranged in age from 9.8 months to 9.8 years old. The major efficacy outcome was the increase in neutrophil CD18 and CD11a surface expression at Month 12, sustained through Month 24 post-infusion. In patients with baseline CD18 <2%, median CD18 expression rose to 54% at Month 12 (range: 20% to 87%).
Estimated Cost Per Month	The cost is unknown, but it is expected to be >$3,000,000 for the drug alone with anticipated ancillary charges.
Associated Benefit	Medical

HMConnects™

As part of the HMConnects cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing, and collaborating on the content of current policies, monitoring trends, and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies, and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com.**

References: [1]Kresladi [package insert]. Cranbury, NJ: Rocket Pharmaceuticals, Inc., March 2026; [2]FDA Approves First Gene Therapy for Severe Leukocyte Adhesion Deficiency Type I, News Release, U.S. Food and Drug Administration, March 27, 2026, available at: https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-therapy-severe-leukocyte-adhesion-deficiency-type-i, accessed April 21, 2026; [3]Kresladi, available at: https://www.kresladi.com/, accessed April 21, 2026; [4]Bondarenko AV, Boyarchuk OR, Sakovich IS, et al. Variable CD18 expression in a 22-year-old female with leukocyte adhesion deficiency I: Clinical case and literature review. Clin Case Rep. 2023;11:e7791; [5]Rocket Pharmaceuticals Wins FDA Accelerated Approval for KRESLADI Gene Therapy in Severe Pediatric LAD-I. American Pharmaceutical Review, available at: https://www.americanpharmaceuticalreview.com/1315-News/624854-Rocket-Pharmaceuticals-Wins-FDA-Accelerated-Approval-for-KRESLADI-Gene-Therapy-in-Severe-Pediatric-LAD-I/, accessed April 21, 2026; [6]Rare Daily Staff, FDA Grants Accelerated Approval to Rocket's Gene Therapy for LAD-1, Global Genes, March 27, 2026, available at: https://globalgenes.org/raredaily/fda-grants-accelerated-approval-to-rockets-gene-therapy-for-lad-1/, accessed April 20, 2026; [7]Kresladi, ClinicalTrials.gov Identifier: NCT03812263, available at: https://clinicaltrials.gov/study/NCT03812263, accessed April 21, 2026.

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