Reference number(s)
7460-A

Specialty Guideline Management Kresladi

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Kresladi	marnetegragene autotemcel

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

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 the ITGB2 gene 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 for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Genetic testing results demonstrating a pathogenic variant in the ITGB2 gene.
- Chart notes, medical records, or lab results documenting the following, if applicable:
    - Flow cytometry showing neutrophil CD18 expression
    - Flow cytometry showing neutrophil CD11a or CD11b expression and clinical history or known family history consistent with leukocyte adhesion deficiency-I (LAD-I)
    - Prior significant bacterial or fungal infection
    - Family history of LAD-I
    - Baseline hepatic, renal, and pulmonary assessments

Prescriber Specialties

This medication must be prescribed by or in consultation with an immunologist, hematologist, or physician who specializes in the treatment of leukocyte adhesion deficiency-I (LAD-I).

Coverage Criteria

Severe Leukocyte Adhesion Deficiency-I (LAD-I)[1-3]

Authorization of 3 months for a one-time administration may be granted for treatment of severe leukocyte adhesion deficiency-I (LAD-I) when all of the following criteria are met:

- Member is 9 months to less than 10 years of age.
- Member has a diagnosis of severe leukocyte adhesion deficiency-I (LAD-I) confirmed by the presence of biallelic pathogenic variants in the ITGB2 gene and one of the following:
    - Flow cytometry indicating CD18 expression on less than 2% of neutrophils [polymorphonuclear neutrophils (PMNs)], or
    - Flow cytometry indicating CD18 expression on 2% or greater of PMNs and both of the following:
        - CD11a or CD11b expression on less than 2% of PMNs
        - Clinical history or known family history consistent with LAD-I
- Member meets one of the following:
    - Member has had at least one prior significant bacterial or fungal infection, or
    - Member has documented family history of LAD-I
- Member is an appropriate candidate for hematopoietic stem cell transplant (HSCT) and has no medically-eligible human leukocyte antigen (HLA)-matched sibling donor.
- Member has a negative serology test for human immunodeficiency virus 1 and 2 (HIV-1/HIV-2) and human T-lymphocytic virus 1 and 2 (HTLV-1/HTLV-2).
- Member will be assessed and monitored for the following, as directed in the manufacturer's prescribing information:
    - Signs and symptoms of infection

    - Signs and symptoms of veno-occlusive disease
    - Neutrophil counts
    - Platelet counts and bleeding
    - Hematologic malignancies
    - Hypersensitivity reactions
- Member does not have evidence of hepatic dysfunction, defined as:
    - Bilirubin greater than 1.5 times the upper limit of normal
    - Alanine aminotransferase (ALT) or aspartate aminotransferase (AST) greater than 2.5 times the upper limit of normal
- Member does not have evidence of renal dysfunction (e.g., eGFR less than 60 ml/min/1.73 m^2) or requirement for dialysis or hemodialysis.
- Member does not have evidence of pulmonary dysfunction, defined as
    - Need for supplemental oxygen within two weeks of initiating therapy with the requested medication
    - Oxygen saturation less than 90% (by pulse oximetry)
- Member does not have evidence of active metastatic or locoregionally advanced malignancy (including hematologic malignancy).
- Member is not infected with persistent bloodstream pathogens or other serious, untreated infections.
- Member does not have any medical or other contraindication for leukapheresis, bone marrow harvest, or conditioning procedures, in the opinion of the provider.
- Member does not have evidence of significant medical conditions [e.g., poorly controlled diabetes, poorly controlled hypertension, poorly controlled cardiac arrythmia, congestive heart failure; or arterial thromboembolic events (including stroke or myocardial infarction)] within 6 months of initiating treatment with the requested medication.
- Member has not received Kresladi or any other gene therapy previously.

References

1. Kresladi [package insert]. Cranbury, NJ: Rocket Pharmaceuticals, Inc.; March 2026.
2. Booth C, Sevilla J, Almarza E, et al. Lentiviral Gene Therapy for Severe Leukocyte Adhesion Deficiency Type 1. *N Engl J Med*. 2025;392(17):1698-1709. doi:10.1056/NEJMoa2407376
3. Justiz Vaillant AA, Ahmad F. Leukocyte Adhesion Deficiency. [Updated 2023 Jul 3]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2026 Jan-. Available from: https://www.ncbi.nlm.nih.gov/books/NBK539770/