



Nonprofit corporations and independent licensees  
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

**P&T Date: 06/11/2026**

**Kresladi™ (marnetegrane autotemcel)**

**HPCPS: J3590**

**Policy:**

*Requests must be supported by submission of chart notes and patient specific documentation.*

- A. Coverage of the requested drug is provided when all the following are met:
  - a. Coverage of the requested drug is considered investigational/experimental for all indications due to insufficient evidence of a clinical benefit
    - i. BCBSM and BCN are awaiting the results of ongoing clinical trials to provide evidence of a clinical benefit

\*\*\*Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

**Background Information:**

- Leukocyte adhesion deficiency-I (LAD-I) is a rare, autosomal recessive pediatric immunodeficiency disorder involving both B and T cells and is characterized by an inability of leukocytes to migrate to the site of infection to kill offending microbes, predisposing patients to recurrent and fatal infections. LAD-I is caused by mutations in the ITGB2 gene encoding for the beta-2 integrin component of CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections.
- During infancy, patients with LAD-I suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Characteristics of patients with LAD-I include: delayed separation of the umbilical cord, recurrent pyogenic infections, with onset in the first weeks of life, infections caused mainly by *Staphylococcus aureus* and *Pseudomonas aeruginosa*, absent pus formation, and periodontitis. Children who survive infancy experience recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia.

This policy and any information contained herein is the property of Blue Cross Blue Shield of Michigan and its subsidiaries, is strictly confidential, and its use is intended for the P&T committee, its members and BCBSM employees for the purpose of coverage determinations.

- There are two phenotypes of LAD-I directly related to the degree of CD18 deficiency: severe deficiency and moderate deficiency. Severe LAD-I is seen in patients with less than 2% of the normal surface expression of CD18. Without a successful bone marrow transplant, survival beyond childhood is rare. Moderate LAD-I is seen in patients with some surface expression of CD18 (variably characterized as 2 to 30% of normal).
- LAD-I is estimated to impact an estimated 800 to 1,000 individuals in the U.S. and Europe. 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. Currently the only potential curative treatment is an allogeneic hematopoietic stem cell transplant (HSCT).
- Management of LAD-I depends on the clinical severity. Mild-to-moderate disease usually responds to antibiotic therapy, while severe disease requires bone marrow transplantation or HSCT. The main aspect of treatment is antibiotic therapy to treat the repeated, characteristic infections associated with the LAD syndrome disorders. Prompt antibiotic therapy is essential during acute infectious episodes.
- Kresladi is an autologous hematopoietic stem cell (HSC)-based gene therapy indicated for the treatment of pediatric patients with severe 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).
- The efficacy of Kresladi was evaluated in one open-label, single-arm, multicenter study in 9 pediatric patients with molecularly confirmed ITGB2-associated severe LAD-I. Severe LAD-I was defined as having neutrophil CD18 expression <2% or CD11a and/or CD11b expression <2% (if neutrophil CD18 expression ≥2%), documented biallelic ITGB2 mutations, and clinical history consistent with severe LAD-I or a known family history. Patients with an available HLA-identical sibling donor for allogeneic HSCT were excluded.
  - The surrogate endpoints for efficacy included improvement in CD18 and CD11a surface expression in neutrophils at Month 12 and sustained expression through Month 24 post-infusion.
  - Seven out of 9 patients had baseline neutrophil CD18 expression <2% and thus were evaluable for post-treatment CD18 assessment. CD18 expression increased in all 7 patients after Kresladi infusion with median CD18 surface expression at Month 12 and Month 24 post-infusion of 54% (range: 20% to 87%) and 50% (range: 16% to 82%), respectively. Neutrophil CD18 expression was sustained through at least Month 42 post-infusion in all 7 patients.
  - Neutrophil CD11a surface expression increased after Kresladi infusion in all 9 patients. Median CD11a surface expression at Month 12 and Month 24 post-infusion were 45% (range: 18% to 75%) and 39% (range: 17% to 65%), respectively. Neutrophil CD11a surface expression was sustained through at least Month 42 post-infusion in all 9 patients.
  - Increases in neutrophil CD18 and CD11a cell surface expression reflect improved function of a protein complex of the two biomarkers on the surface of neutrophils which is used as a surrogate endpoint that is reasonably likely to predict clinical benefit in LAD-I for accelerated approval. The clinical benefit of Kresladi will be confirmed in patients with severe LAD-I through post-marketing requirements. Rocket Pharmaceuticals stated that FDA guidance on the path from accelerated to full approval involves the continued long-term follow-up of the original clinical trial and evaluation of real-world experience with Kresladi.

- The plan considers cellular and gene therapies to be proven effective to treat a condition if the following are true: the drug has full FDA approval that was not obtained under the FDA's accelerated approval pathway and the drug met all of its primary endpoint(s) in its pivotal trials, or if confirmatory trials were required, the drug met its primary endpoint(s) in its confirmatory trial(s). Cellular and gene therapies that haven't been proven effective (do not meet the listed criteria) are considered a benefit contract exclusion for most plans.
- Kresladi was approved under the FDA's accelerated approval pathway, and therefore, is considered experimental/investigational until further confirmatory trials are complete and the medication gets full FDA approval.

## References:

1. Kresladi [prescribing information]. Cranbury NJ: Rocket Pharmaceuticals, Inc.; March 2026.
2. Rocket Pharma News Release Details. Rocket Pharmaceuticals Announces FDA Approval of KRESLADI™ for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I). March 27, 2026  
<https://ir.rocketpharma.com/news-releases/news-release-details/rocket-pharmaceuticals-announces-fda-approval-kresladitm/>
3. U.S. Food & Drug Administration. FDA News Release. FDA approves first gene therapy for severe leukocyte adhesion deficiency type I. March 26, 2026. <https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-therapy-severe-leukocyte-adhesion-deficiency-type-i>
4. Justiz Vaillant AA, Ahmad F. Leukocyte Adhesion Deficiency. [Updated 2023 Jul 3]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2023 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK539770>
5. National Library of Medicine. A clinical trial to evaluate the safety and efficacy of RP-L201 in subjects with leukocyte adhesion deficiency-I. ClinicalTrials.gov. Study Details | A Clinical Trial to Evaluate the Safety and Efficacy of RP-L201 in Subjects With Leukocyte Adhesion Deficiency-I | ClinicalTrials.gov
6. Etzioni A, Leukocyte-adhesion deficiency. UpToDate. Literature current through Dec.2023; accessed January 2024. <https://www.uptodate.com/contents/leukocyte-adhesion-deficiency?search=leukocyte>
7. Johnson V. Rocket's LAD-I gene therapy BLA accepted for priority review. CGTlive. October 2, 2023. <https://www.cgtlive.com/view/rocket-lad-i-gene-therapy-bla-rp-l201-priority-review>

| Policy History   |                                                   |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
|------------------|---------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------|---------------------------------------------------|------|-----|-----|-----|-------|-----|------|-----|
| #                | Date                                              | Change Description                                                                                                                                                                                                                                                                                         |                  |                                                   |      |     |     |     |       |     |      |     |
| 1.2              | Effective Date:<br>06/11/2026                     | New Drug Review                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| 1.1              | Effective Date:<br>05/01/2026                     | UM medical management system update for MAPPO and BCNA <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table> | Line of Business | PA Required in Medical Management System (Yes/No) | BCBS | Yes | BCN | Yes | MAPPO | Yes | BCNA | Yes |
| Line of Business | PA Required in Medical Management System (Yes/No) |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCBS             | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCN              | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| MAPPO            | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCNA             | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| 1.0              | Effective Date:<br>04/06/2026                     | UM medical management system update for BCBS and BCN <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>     | Line of Business | PA Required in Medical Management System (Yes/No) | BCBS | Yes | BCN | Yes | MAPPO | No  | BCNA | No  |
| Line of Business | PA Required in Medical Management System (Yes/No) |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCBS             | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCN              | Yes                                               |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| MAPPO            | No                                                |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |
| BCNA             | No                                                |                                                                                                                                                                                                                                                                                                            |                  |                                                   |      |     |     |     |       |     |      |     |

\* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.

# Blue Cross Blue Shield/Blue Care Network of Michigan

## Medication Authorization Request Form



This form is to be used by participating physicians to obtain coverage for **drugs covered under the medical benefit**. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

Nonprofit corporations and independent licensees of the Blue Cross and Blue Shield Association

| PATIENT INFORMATION                                                         | PHYSICIAN INFORMATION                                       |
|-----------------------------------------------------------------------------|-------------------------------------------------------------|
| <b>Name</b>                                                                 | <b>Name</b>                                                 |
| <b>ID Number</b>                                                            | <b>Specialty</b>                                            |
| <b>D.O.B.</b> <input type="checkbox"/> Male <input type="checkbox"/> Female | <b>Address</b>                                              |
| <b>Diagnosis</b>                                                            | <b>City /State/Zip</b>                                      |
| <b>Drug Name</b>                                                            | <b>Phone/Fax: P: (       )       - F: (       )       -</b> |
| <b>Dose and Quantity</b>                                                    | <b>NPI</b>                                                  |
| <b>Directions</b>                                                           | <b>Contact Person</b>                                       |
| <b>Date of Service(s)</b>                                                   | <b>Contact Person Phone / Ext.</b>                          |

### STEP 1: DISEASE STATE INFORMATION

- Is this request for: ☐ Initiation ☐ Continuation *Date patient started therapy:* \_\_\_\_\_
- Administered by patient or a medical professional? ☐ patient (self) ☐ health care professional (physician, nurse, etc.)
- Site of administration? ☐ Provider office/Home infusion ☐ Other: \_\_\_\_\_  
☐ Hospital outpatient facility (go to #4) *Reason for Hospital Outpatient administration:* \_\_\_\_\_  
☐ Hospital inpatient facility for Car-T therapy only (for example: Kymriah, Yescarta, or Tecartus) (go to #5)
- Please specify location of administration if hospital outpatient infusion: \_\_\_\_\_
- Please specify location of administration if hospital inpatient infusion: \_\_\_\_\_
- Please provide the NPI number for the place of administration: \_\_\_\_\_
- Initiation AND Continuation of therapy:**
  - What is the patient's diagnosis? \_\_\_\_\_
  - What other medication has the patient received for their condition? Please list \_\_\_\_\_  
    - Please describe the response to previous therapies: \_\_\_\_\_
  - Will the patient be receiving any other treatment for the listed condition while on this medication? Please list: \_\_\_\_\_
  - Please list any labs values important for diagnosing or monitoring this patient's condition: \_\_\_\_\_
- Continuation of therapy:**
  - Has the patient progressed while on this medication? ☐ yes ☐ no
  - How has the patient's condition changed while on this medication?
 

☐ Improved: Please describe: \_\_\_\_\_
 ☐ Stable: please describe: \_\_\_\_\_
 ☐ Worsened; Please describe: \_\_\_\_\_
 ☐ Other; Please describe: \_\_\_\_\_

*Chart notes are required for the processing of all requests. Please add any other supporting medical information necessary for our review (required)*

**Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.**

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

|                             |                                                                                                     |                                                                                             |
|-----------------------------|-----------------------------------------------------------------------------------------------------|---------------------------------------------------------------------------------------------|
| <b>Physician's Name</b>     | <b>Physician Signature</b>                                                                          | <b>Date</b>                                                                                 |
| <b>Step 2:</b><br>Checklist | <input type="checkbox"/> Form Completely Filled Out<br><input type="checkbox"/> Provide chart notes | <input type="checkbox"/> Attach test results                                                |
| <b>Step 3:</b><br>Submit    | <b>By Fax: BCBSM Specialty Pharmacy Mailbox</b><br>1-877-325-5979                                   | <b>By Mail: BCBSM Specialty Pharmacy Program</b><br>P.O. Box 312320, Detroit, MI 48231-2320 |

**Confidentiality notice:** This transmission contains confidential information belonging to the sender that is legally privileged. This information is intended only for use of the individual or entity named above. The authorized recipient of this information is prohibited from disclosing this information to any other party. If you are not the intended recipient, you are hereby notified that any disclosure, copying, distribution or action taken in reliance on the contents of this document is strictly prohibited. If you have received this in error, please notify the sender to arrange for the return of this document.

7/26/2018, 9/18/2018; 1/31/2020; 3/17/2020; 8/9/2021