 Draft Revision Policy: Do Not Implement

Atidarsagene Autotemcel (LENMELDY™)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

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

Lenmeldy is indicated for the treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD).

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:
Chart notes, medical records, or lab results documenting all of the following:

- Pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ), or early symptomatic early juvenile (ESEJ) classification of metachromatic leukodystrophy (MLD).
- Variant(s) in the ARSA gene.
- Deficiency of arylsulfatase A (ARSA) activity on biochemical testing.
- Elevated urine sulfatide levels, if applicable.
- Medical records (e.g., chart notes and/or laboratory reports) documenting baseline liver function (e.g., LFT) and renal function (e.g., eGFR).

PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metachromatic leukodystrophy (MLD).

COVERAGE CRITERIA

Metachromatic Leukodystrophy (MLD)

Authorization of 3 months for a one-time administration may be granted for treatment of metachromatic leukodystrophy (MLD) when all of the following criteria are met:

- Member must have a diagnosis of one of the following types of MLD:

  - Pre-symptomatic late infantile (PSLI), confirmed by at least two of the following:
    - Age of onset of symptoms in the older sibling(s) ≤30 months.
    - Two null (0) variant arylsulfatase A (ARSA) allele(s).
    - Peripheral neuropathy at electroneurographic study.
  - Pre-symptomatic early juvenile (PSEJ), confirmed by at least two of the following:
    - Age of onset of symptoms (in the member or in the older sibling) between 30 months and 6 years (inclusive).
    - One null (0) and one residual (R) variant ARSA allele(s).
    - Peripheral neuropathy at ~~electrographic~~ electroneurographic study.
  - Early symptomatic early juvenile (ESEJ), confirmed by Gross Motor Function Classification-MLD (GMFC-MLD) score of 0-1, Intelligence quotient (IQ) of ≥ 85, and at least two of the following:
    - Age of onset of symptoms (in the member or in the older sibling) between 30 months and 6 years (inclusive).
    - One null (0) and one residual (R) variant ARSA allele(s).
    - Peripheral neuropathy at ~~electrographic~~ electroneurographic study.
- The diagnosis was confirmed by all of the following:
  - Biochemical testing documenting ARSA activity below the normal range for the laboratory performing the test.
  - The presence of two disease-causing ARSA alleles, either known or novel variants, identified on genetic testing.
  - If novel variants are identified, testing showing elevated urine sulfatide levels.
- Member does not have a diagnosis of late juvenile form of MLD.
- Member has not received Lenmeldy or any other gene therapy previously.
- Member does not have evidence of residual cells of donor origin if the member has received a prior allogeneic hematopoietic stem cell transplant (allo-HSCT) and has not received allo-HSCT in the past 6 months.
- Member has a negative serology test for human immunodeficiency virus 1 and 2 (HIV-1/HIV-2), hepatitis B (HBV), hepatitis C (HCV), human T-lymphocytic virus 1 and 2 (HTLV-1/HTLV-2), and mycoplasma infection.
- Member's thrombotic risk and need for anti-thrombotic treatment have been assessed at baseline and will be monitored after Lenmeldy administration as clinically appropriate.
- Member is not affected by neoplastic diseases.
- Member is not affected by cytogenic alterations typical of myelodysplastic syndrome or acute myelogenous leukemia.
- Member will be monitored for hematologic malignancies annually (e.g., complete blood count with differential) and integration site analysis as warranted for at least 15 years after treatment with Lenmeldy.
- Member's liver function (e.g., LFT) and renal function (e.g., eGFR) have been assessed at baseline and will be monitored after Lenmeldy administration as clinically appropriate.

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice

Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Lenmeldy [package insert]. Boston, MA: Orchard Therapeutics North America.; March 2024.
2. Gomez-Ospina N. Arylsulfatase A Deficiency. 2006 May 30 [Updated 2024 Apr 25]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1130/. Accessed January 28, 2026.

EFFECTIVE DATE

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