Reference number(s)
6441-A

Standard Medicare Part B Management Lenmeldy

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
Lenmeldy	atidarsagene 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]

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 will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

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

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- 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.

Coverage Criteria

Metachromatic Leukodystrophy (MLD)[1,2]

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).
  - Pre-symptomatic early juvenile (PSEJ).
  - Early symptomatic early juvenile (ESEJ).
- The diagnosis was confirmed by all of the following:
  - Biochemical testing documenting ARSA activity that is 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 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).

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Lenmeldy.
- The available compendium
  - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
  - Micromedex DrugDex
  - American Hospital Formulary Service- Drug Information (AHFS-DI)
  - Lexi-Drugs
  - Clinical Pharmacology
- Arylsulfatase A Deficiency - Gene Reviews.

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After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Lenmeldy are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing 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; 1993-2024. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1130/. Accessed January 23, 2025.

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