blue california
Promise Health Plan

atidarsagene autotemcel (Lenmeldy)

Medical Benefit Drug Policy

Place of Service
Hospital Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT
Mechanism of Action: Autologous hematopoietic stem cell-based gene therapy
HCPCS:
J3391:Injection, atidarsagene autotemcel, per treatment
How Supplied:
NDC: 83222-0200-1
Up to eight 50 mL infusion bags (containing 1.8 to 11.0 x10^6 CD34+ cells/mL), overwrap, and metal cassette

Condition(s) listed in policy *(see coverage criteria for details)*

- Metachromatic Leukodystrophy (MLD)

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the California Code of Regulations (CCR), Title 22, Section 51303 and 51313 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

Coverage Criteria
The following condition(s) require Prior Authorization/Preservice.

Metachromatic Leukodystrophy (MLD)
Meets medical necessity if all the following are met:

1. A diagnosis of MLD as confirmed by gene sequencing and/or deletion/duplication assessment identifies the presence of biallelic arylsulfatase-A (ARSA) pathogenic variant or likely pathogenic variants.
2. If a proband individual, has all of the following:
   a. Elevated urinary excretion of sulfatides in a 24-hour urine collection
   b. Deficient ARSA enzyme activity (<10% of normal values) in peripheral blood mononuclear cells or fibroblasts

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3. Patient has not received treatment with hematopoietic stem cell transplant within the last six months
4. Meets ONE of the following:
   a. Presymptomatic late infantile MLD (PSLI-MLD)
   b. Presymptomatic early juvenile MLD (PSEJ-MLD)
   c. Early symptomatic early juvenile MLD (ESEJ-MLD) as defined by all the following:
      i. Gross Motor Function Classification (GMFC-MLD) level ≤1
      ii. Intelligence quotient (IQ) ≥85
5. Patient is clinically stable and meets the institutional requirements for a stem cell transplant. The requirements may include the following:
   a. Adequate performance status score (e.g. Karnofsky performance status, Lansky performance status)
   b. Absence of advanced liver disease
   c. Adequate estimate glomerular filtration rate (eGFR)
   d. Adequate diffusing capacity of the lungs for carbon monoxide (DLCO)
   e. Adequate ventricular ejection fraction (LVEF)
   f. Absence of clinically significant active infection(s)

Table 1. Summary of eligible MLD subtypes for Lenmeldy

MLD Subtype	Patient Age at Disease Onset/Diagnosis	Presentation/Symptoms at the Time of Request
PSLI-MLD	Age <30 months	Absence of neurological signs and symptoms of MLD, with or without signs of the disease revealed by electroneurographic and brain magnetic resonance imaging.
PSEJ-MLD	30 months ≤ Age < 7 years	
ESEJ-MLD	30 months ≤ Age < 7 years	• Able to walk independently without support (i.e., GMFC-MLD level ≤1) • Normal cognitive function (i.e., IQ ≥85)

Not covered for late juvenile MLD (LJ-MLD) and adult MLD

Covered Doses:

- Lenmeldy is composed of one to eight infusion bags which contain 2 to 11.8× 10^6 cells/mL (1.8 to 11.8 x 10^6 CD34+ cells/ml) suspended in cryopreservation solution
- Lenmeldy is a single-dose cell suspension for intravenous infusion.

Coverage Period:
One-time treatment per lifetime

ICD-10:
E75.25

References

1. Lenmeldy (atidarsagene autotemcel) Prescribing Information. Orchard Therapeutics North America, Boston, MA: 3/2024.
2. Wang RY, Bodamer OA, Watson MS, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011; 13(5): 457-484

Review History

Date of Last Annual Review: 2Q2025
Changes from previous policy version:

- HCPCS: Added J3391, effective 7/1/25.

Blue Shield of California Medication Policy to Determine Medical Necessity Reviewed by P&T Committee