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Section:	Prescription Drugs	**Effective Date:**	October 1, 2024
Subsection:	Hematological Agents	**Original Policy Date:**	June 21, 2024
Subject:	Lenmeldy	**Page:**	1 of 4

Last Review Date: September 6, 2024

Lenmeldy

Description

Lenmeldy (atidarsagene autotemcel)

Background
Metachromatic leukodystrophy (MLD) is a rare lysosomal storage disease caused by deficient activity of arylsulfatase A. It is an autosomal recessive condition and results in a buildup of sulfatides that leads to the destruction of the myelin sheath, leading to progressive demyelination of the central and peripheral nervous system. MLD is a serious condition and causes death within 5-6 years in early-onset form (1).

Lenmeldy (atidarsagene autotemcel) inserts one or more functional copies of the human arylsulfatase A (ARSA) complementary deoxyribonucleic acid (cDNA) into the patients' hematopoietic stem cells (HSCs). After Lenmeldy infusion, transduced CD34+ HSCs engraft in bone marrow, repopulate the hematopoietic compartment and their progeny produce ARSA enzyme. Functional ARSA enzyme can breakdown or prevent the harmful accumulation of sulfatides (2).

Regulatory Status
FDA-approved indication: Lenmeldy is an autologous hematopoietic stem cell-based gene therapy 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) (2).

Lenmeldy contains warnings for thrombosis and thromboembolic events, encephalitis, serious infections, veno-occlusive disease, delayed platelet engraftment, risk of neutrophil engraftment failure, risk of insertional oncogenesis, and risk of hypersensitivity reactions (2).

The safety and effectiveness of Lenmeldy have not yet been established in children with the late juvenile form of MLD (2).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Lenmeldy may be considered **medically necessary** if the conditions indicated below are met.

Lenmeldy may be considered **investigational** for all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

1. Metachromatic leukodystrophy (MLD)

AND ALL of the following:

a. Diagnosis of MLD has been confirmed by **ALL** of the following:
   i. Genetic test showing biallelic ARSA pathogenic variants
   ii. ARSA enzyme activity in leukocytes below normal limits
   iii. Elevated sulfatide levels in urine
b. Patient has **ONE** of the following subtypes of MLD:
   i. Pre-symptomatic late infantile (PSLI)
   ii. Pre-symptomatic early juvenile (PSEJ)
   iii. Early symptomatic early juvenile (ESEJ)
c. **NO** clinically significant bacterial, fungal, parasitic, or viral infection
d. **NO** prior gene therapy or allogenic hematopoietic stem cell transplant

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre – PA Allowance

None

Prior - Approval Limits

Quantity One infusion (only one PA approval for one infusion per lifetime)

Rationale

Summary

Lenmeldy is an autologous hematopoietic stem cell-based gene therapy for the treatment of children with metachromatic leukodystrophy (MLD) who are pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ). The safety and effectiveness of Lenmeldy have not yet been established in children with the late juvenile form of MLD (2).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Lenmeldy while maintaining optimal therapeutic outcomes.

References

1. Lamichhane A, Rocha Cabrero F. Metachromatic Leukodystrophy. National Institutes of Health. Available from: https://www.ncbi.nlm.nih.gov/books/NBK560744/.
2. Lenmeldy [package insert]. Boston, MA: Orchard Therapeutics North America; March 2024.

Policy History

Date	Action
June 2024	Addition to PA
September 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 6, 2024 and is effective on October 1, 2024.