

Medical Policy



Title: Lenmeldy (atidarsagene autotemcel)

Professional / Institutional
Original Effective Date: September 1, 2024
Latest Review Date: December 26, 2025
Current Effective Date: December 26, 2025

State and Federal mandates and health plan member contract language, including specific provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage. To verify a member's benefits, contact [Blue Cross and Blue Shield of Kansas Customer Service](#).

The BCBSKS Medical Policies contained herein are for informational purposes and apply only to members who have health insurance through BCBSKS or who are covered by a self-insured group plan administered by BCBSKS. Medical Policy for FEP members is subject to FEP medical policy which may differ from BCBSKS Medical Policy.

The medical policies do not constitute medical advice or medical care. Treating health care providers are independent contractors and are neither employees nor agents of Blue Cross and Blue Shield of Kansas and are solely responsible for diagnosis, treatment and medical advice.

If your patient is covered under a different Blue Cross and Blue Shield plan, please refer to the Medical Policies of that plan.

POLICY AGENT SUMMARY – MEDICAL PRIOR AUTHORIZATION

Indication	Dose												
Metachromatic Leukodystrophy (MLD)	Lenmeldy is provided as a single dose for infusion containing a suspension of CD34+ cells in one to eight infusion bags. The minimum and maximum recommended dose is based on the MLD disease subtype. <table><tr><th>MLD Subtype</th><th>Minimum Recommended Dose (CD34+ cells/kg)</th><th>Maximum Recommended Dose (CD34+ cells/kg)</th></tr><tr><td>Pre-symptomatic late infantile</td><td>4.2 x 10⁶</td><td>30 x 10⁶</td></tr><tr><td>Pre-symptomatic early juvenile</td><td>9 x 10⁶</td><td>30 x 10⁶</td></tr><tr><td>Early symptomatic early juvenile</td><td>6.6 x 10⁶</td><td>30 x 10⁶</td></tr></table>	MLD Subtype	Minimum Recommended Dose (CD34+ cells/kg)	Maximum Recommended Dose (CD34+ cells/kg)	Pre-symptomatic late infantile	4.2 x 10 ⁶	30 x 10 ⁶	Pre-symptomatic early juvenile	9 x 10 ⁶	30 x 10 ⁶	Early symptomatic early juvenile	6.6 x 10 ⁶	30 x 10 ⁶
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Indication	Dose
	The dose administered is calculated based on the child's weight at time of Lenmeldy infusion using the information provided on the Lot Information Sheet. See the Lot Information Sheet provided with the product shipment for additional information pertaining to dose.
<i>– Lenmeldy is for autologous use only. The patient's identity must match the patient identifiers on the drug cassette(s) and infusion bag(s).</i> <i>– Mobilization, apheresis, and myeloablative conditioning are required prior to LENMELDY infusion. Before initiating these procedures, confirm that hematopoietic stem cell (HSC) gene therapy is appropriate for the child.</i> <i>– A collection of a minimum of 8.0×10^6 CD34+ cells/kg of autologous cells is required based on a weight at time of apheresis collection. Collection of the minimum number of CD34+ cells required for manufacture may be achieved using one or more cycles of mobilization. A collection of unmanipulated back-up CD34+ cells of at least 2.0×10^6 CD34+ cells/kg is required. These cells must be collected from the child and be cryopreserved prior to myeloablative conditioning.</i>	

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

I. Length of Authorization

Coverage will be provided for one treatment course (1 dose) and may not be renewed.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 1 billable unit for one dose

III. Initial Approval Criteria ¹

- Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is **REQUIRED** on all requests for authorizations. Records will be reviewed at the time of submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax. Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines.*

Coverage is provided for the following conditions:

- Patient age is less than 18 years; **AND**
- Patient is screened and found to be negative for hepatitis B virus (HBV), hepatitis C virus (HCV), human T-lymphotrophic virus 1 & 2 (HTLV-1/HTLV-2), human

immunodeficiency virus 1 & 2 (HIV-1/HIV-2), and mycoplasma infection before collection of cells for manufacturing; **AND**

- Patient will not be administered vaccinations during the 6 weeks preceding the start of myeloablative conditioning, and until hematological recovery following treatment (*Note: Where feasible, administer childhood vaccinations prior to myeloablative conditioning*); **AND**
- Patient risk factors for thrombosis as well as veno-occlusive disease have been evaluated prior to administration; **AND**
- Prophylaxis for infection will be followed according to standard institutional guidelines; **AND**
- Patient will be monitored for hematological malignancies periodically after treatment; **AND**
- Patients will not receive prophylactic HIV anti-retroviral therapy for at least one-month preceding mobilization (*Note: anti-retrovirals may interfere with manufacturing*); **AND**
- Patient will have mobilization of stem cells using granulocyte-colony stimulating factor (G-CSF with or without plerixafor); **AND**
- Used as single agent therapy (*Note: not inclusive of busulfan conditioning regimen*); **AND**
- Patient has not received a prior allogeneic stem cell transplant (*or has, but is without evidence of residual donor cells present*), and is a candidate for autologous stem cell transplantation (e.g., adequate renal and hepatic function); **AND**
- Patient has not received other gene therapy for MLD; **AND**

Metachromatic Leukodystrophy (MLD)

- Patient has a confirmed diagnosis of MLD (*also known as arylsulfatase A deficiency*) as evidenced by the following biochemical and molecular markers:
 - Arylsulfatase A (ARSA) enzyme activity below the normal range in peripheral blood mononuclear cells-leukocytes or fibroblasts OR increased urinary excretion of sulfatides; **AND**
 - Presence of biallelic ARSA pathogenic mutation of known polymorphisms (*Note: Patients with novel mutations, a 24-hour urine collection must show elevated sulfatide levels*); **AND**
- Patient has pre-symptomatic late infantile (PSLI), presymptomatic, early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ) disease (*Note: Requests for children with late juvenile form of the disease will be reviewed on a case-by-case basis*)

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Ⓢ Orphan Drug

IV. Renewal Criteria

Duration of authorization has not been exceeded (*refer to Section I*)

Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

CLINICAL RATIONALE

See package insert for FDA pres<https://dailymed.nlm.nih.gov/dailymed/index.cfm>

CODING

The following codes for treatment and procedures applicable to this policy are included below for informational purposes. This may not be a comprehensive list of procedure codes applicable to this policy.

Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

The code(s) listed below are medically necessary ONLY if the procedure is performed according to the "Policy" section of this document.

HCPCS Code:

- J3391 – Injection, atidarsagene autotemcel, per treatment; 1 billable unit = 1 treatment

NDC(s):

- Lenmeldy containing 2 to 11.8× 10⁶ cells/mL (1.8 to 11.8 x 10⁶ CD34+ cells/ml) suspended in one or eight patient-specific infusion bags: 83222-0200-xx

REVISIONS	
Posted 08-01-2024 Effective 09-01-2024	Policy added to the bcbsks.com web site.
12-23-2024	Updated Approval Criteria: • Removed requirement that patient does not have a known 10/10 human leukocyte antigen matched related donor willing to participate in HSCT per trade Medical policy reviewed and maintained by Prime Therapeutics
07-01-2025	Updated Coding Section • Removed J3590

REVISIONS	
	Added new code J3391
Posted: 11-26-2025 Effective: 12-26-2025	Length of Authorization Section - Added: - Coverage will be provided for one treatment course (1 dose) and may not be renewed. - Removed: - One Treatment course (1 dose) and cannot be renewed
	Dosing Limits Section - Added: - Max Units (per dose and over time) [HCPCS Unit]: 1 billable unit for one dose - Removed: A. Quantity Limit (max daily dose) [NDC Unit]: - A single dose of Lenmeldy contains 2 to 11.8×10^6 cells/mL (1.8 to 11.8×10^6 CD34+ cells/mL) suspended in one or more patient-specific infusion bags B. Max Units (per dose and over time) [HCPCS Unit]: - A single dose of Lenmeldy contains 2 to 11.8×10^6 cells/mL (1.8 to 11.8×10^6 CD34+ cells/mL) suspended in one to eight patient-specific infusion bags
	Initial Approval Criteria Section - Added: "Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax. Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines." - Removed: "Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax."
	Renewal Criteria Section - Added: - Duration of authorization has not been exceeded (<i>refer to Section I</i>) - Removed: - Coverage cannot be renewed.
	HCPCS Code: - Added: - Added: 1 billable unit = 1 treatment to the nomenclature for J3391
	Medical Policy is maintained by Prime Therapeutics, LLC

REFERENCES

1. Lenmeldy [package insert]. Boston, PA; Orchard Therapeutics NA; April 2024. Accessed April 2024.
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10. Bonkowsky JL (Mar 2024). Metachromatic leukodystrophy. In Patterson MC, Firth HV, Dashe JF (Eds.). *UpToDate*. Accessed March 20, 2024. Available from: https://www.uptodate.com/contents/metachromatic-leukodystrophy?search=Metachromatic%20Leukodystrophy%20&source=search_result&selectedTitle=1%7E33&usage_type=default&display_rank=1#H6
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