Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 328.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Lenmeldy (atidarsagene autotemcel)

Applicable line of business:

Commercial	X	Medicaid	X
Medicare	X	ACA	X
CHIP	X		

I. Policy:
Lenmeldy (atidarsagene autotemcel)

II. Purpose/Objective:
To provide a policy of coverage regarding Lenmeldy (atidarsagene autotemcel)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than the department requiring/authoring the policy.
3. Devised – the date the policy was implemented.
4. Revised – the date of every revision to the policy, including typographical and grammatical changes.
5. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:
Lenmeldy (atidarsagene autotemcel) is a gene therapy consisting of autologous CD34+ hematopoietic cells transduced with a lentiviral vector encoding the human arylsulfatase A (ARSA) gene to add functional copies of the ARSA cDNA into the hematopoietic stem cells. After atidarsagene autotemcel infusion, transduced CD34+ hematopoietic stem cells engraft in the bone marrow and repopulate the hematopoietic compartment and their progeny produce ARSA enzyme. Functional ARSA breaks down or prevents the harmful accumulation of sulfatides.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Lenmeldy (atidarsagene autotemcel) will be considered medically necessary for all lines of business when ALL of the following criteria are met:

Pre-symptomatic Late Infantile Metachromatic Leukodystrophy (PSLI MDL)
- Prescription written by a hematologist, oncologist, and/or stem cell transplant specialist **AND**
- Medical record documentation of **two** out of **three** of the following:
    - Age at onset of symptoms in older sibling(s) ≤ 30 months
    - Presence of two null (0) mutant arylsulfatase A (ARSA) alleles
    - Peripheral neuropathy as determined by electroneurographic study

  AND
- Medical record documentation that the patient does not have disease-related symptoms (motor or cognitive symptoms)* **AND**
- Medical record documentation of laboratory results (including name of testing laboratory, reference range, and resulting value) demonstrating ARSA activity below the normal range in peripheral blood mononuclear cells or fibroblasts (defined by a resulting value less than the testing laboratory's lower limit of normal) **AND**
- Medical record documentation of the presence of sulfatides in a 24-hour urine collection **AND**
- Medical record documentation that the member has a negative serology test for Human Immunodeficiency Virus (HIV), Hepatitis B, and Hepatitis C **AND**
- Medical record documentation that the member has not had a prior hematopoietic stem cell transplant or hematopoietic stem-cell based gene therapy

*Note to Reviewer: In the clinical trial, pre-symptomatic was defined as patients without neurological impairment (disease-related symptoms) with or without signs of the disease revealed by instrumental evaluations (electroneurographic and brain MRI)

Pre-symptomatic Early Juvenile Metachromatic Leukodystrophy (PSEJ MLD)
- Prescription written by a hematologist, oncologist, and/or stem cell transplant specialist **AND**
- Medical record documentation of **two** out of **three** of the following:
    - Age at onset of symptoms in an older sibling between 30 months and 6 years (has not celebrated 7th birthday)
    - Presence of one null (0) and one residual (R) mutant arylsulfatase A (ARSA) alleles
    - Peripheral neuropathy as determined by electroneurographic study

  AND
- Medical record documentation that the patient does not have disease-related symptoms (motor or cognitive symptoms)* **AND**
- Medical record documentation of laboratory results (including name of testing laboratory, reference range, and resulting value) demonstrating ARSA activity below the normal range in peripheral blood mononuclear cells or fibroblasts (defined by a resulting value less than the testing laboratory's lower limit of normal) **AND**
- Medical record documentation of the presence of sulfatides in a 24-hour urine collection **AND**

- Medical record documentation that the member has a negative serology test for Human Immunodeficiency Virus (HIV), Hepatitis B virus, and Hepatitis C virus **AND**
- Medical record documentation that the member has not had a prior hematopoietic stem cell transplant or hematopoietic stem-cell based gene therapy

*Note to Reviewer: In the clinical trial, pre-symptomatic was defined as patients without neurological impairment (disease-related symptoms) with or without signs of the disease revealed by instrumental evaluations (electroneurographic and brain MRI)

Early Symptomatic Early Juvenile Metachromatic Leukodystrophy (ESEJ MLD)

- Prescription written by a hematologist, oncologist, and/or stem cell transplant specialist **AND**
- Medical record documentation of **two** out of **three** of the following:
    - Age at onset of symptoms (in the patient or in an older sibling) between 30 months and 6 years (has not celebrated 7th birthday)
    - Presence of one null (0) and one residual (R) mutant arylsulfatase A (ARSA) alleles
    - Peripheral neuropathy as determined by electroneurographic study

  AND
- Medical record documentation that the patient has been diagnosed with ESEJ MLD within 6 months of the first reported symptoms (i.e., cognitive symptoms: intelligence quotient ≥ 70 and motor symptoms: the ability to walk independently for ≥ 10 steps) **AND**
- Medical record documentation of laboratory results (including name of testing laboratory, reference range, and resulting value) demonstrating ARSA activity below the normal range in peripheral blood mononuclear cells or fibroblasts (defined by a resulting value less than the testing laboratory's lower limit of normal) **AND**
- Medical record documentation of the presence of sulfatides in a 24-hour urine collection **AND**
- Medical record documentation that the member has a negative serology test for Human Immunodeficiency Virus (HIV), Hepatitis B virus, and Hepatitis C virus **AND**
- Medical record documentation that the member has not had a prior hematopoietic stem cell transplant or hematopoietic stem-cell based gene therapy

Note to Reviewer: In the clinical trial, early symptomatic was initially defined as a patient identified within 6 months from the first reported symptoms. Subsequently, ESEJ patients were defined as meeting both criteria: intelligence quotient ≥ 70 and the ability to walk independently for ≥ 10 steps.

AUTHORIZATION DURATION: 2 months, a one (1) time approval per lifetime; Requests for authorizations exceeding these limits will require the following medical record documentation of peer-reviewed literature citing well-designed clinical trials to indicate that the member's healthcare outcome will be improved by dosing beyond the FDA-approved treatment duration.

LINE OF BUSINESS:
Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Lenmeldy [prescribing information]. Boston, MA: Orchard Therapeutics North America. March 2024. Accessed September 2024

This policy will be revised as necessary and reviewed no less than annually.

Devised: 10/18/24

Revised:

Reviewed:

MA UM Committee approval: 12/31/24

DHS PARP approval: 1/6/25