New Hampshire Medicaid Fee-for-Service Program
Lenmeldy™ (atidarsagene autotemcel) Criteria

Approval Date: June 10, 2024

Medications

Brand Names	Generic Names	Indication
Lenmeldy™	atidarsagene autotemcel	Treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD)

Criteria for Approval

1. Patient is less than 18 years of age; **AND**
2. Confirmed diagnosis of metachromatic leukodystrophy (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:** for patients with novel mutations, a 24-hour urine collection must show elevated sulfatide levels); **AND**
3. 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); **AND**
4. Lenmeldy will be used as single-agent therapy (**Note:** not inclusive of busulfan conditioning regimen); **AND**
5. Patient has **not** received prior allogeneic stem cell transplant; **OR**
6. Patient has received prior allogeneic stem cell transplant, but is without evidence of residual donor cells present; **AND**
7. Patient is a candidate for autologous stem cell transplantation (e.g., adequate renal and hepatic function); **AND**
8. Patient has **not** received other gene therapy for MLD; **AND**

9. Patient has been screened and found to be negative for hepatitis B virus (HBV), hepatitis C virus (HCV), human T-lymphotrophic virus 1 and 2 (HTLV-1/HTLV-2), human immunodeficiency virus 1 and 2 (HIV-1/HIV-2), cytomegalovirus (CMV), and mycoplasma infection before collection of cells for manufacturing; **AND**
10. Patient will have mobilization of stem cells using granulocyte-colony stimulating factor (G-CSF) with or without plerixafor; **AND**
11. Myeloablative conditioning (e.g., busulfan) will be administered at least 24 hours prior to Lenmeldy infusion; **AND**
12. Patient risk factors for thrombosis and veno-occlusive disease have been evaluated prior to administration; **AND**
13. Prophylaxis for infection will be followed according to standard institutional guidelines; **AND**
14. Patient will be monitored for hematological malignancies periodically after treatment; **AND**
15. Patient will **not** receive prophylactic HIV anti-retroviral (ARV) therapy for at least 1-month preceding mobilization (**Note:** ARVs may interfere with manufacturing); **AND**
16. Patient does **not** have a known 10/10 human leukocyte antigen matched related donor willing to participate in allogeneic hematopoietic stem cell transplantation (HSCT); **AND**
17. 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**
18. Females of childbearing potential have a confirmed negative pregnancy test prior to the start of mobilization and negative test is reconfirmed prior to conditioning procedures and before Lenmeldy administration.

Criteria for Denial

Above criteria are not met.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	05/07/2024
Commissioner designee	Approval	06/10/2024