New Hampshire Medicaid Fee-for-Service Program
Skysona® (elivaldogene autotemcel) Criteria

Approval Date: June 10, 2024

Medications

Brand Names	Generic Names	Indication
Skysona®	elivaldogene autotemcel	To slow the progression of neurologic dysfunction in boys 4–17 years of age with early, active cerebral adrenoleukodystrophy (CALD)

Criteria for Approval

1. Patient is a male 4 years of age or older, but less than 18 years of age; **AND**
2. Patient has a documented diagnosis of early, active cerebral adrenoleukodystrophy (CALD) as outlined by the following:
   - Patient has elevated very-long-chain fatty acids (VLCFA) values:
     - C26:0, 1.30 + 0.45 (normal: 0.23 + 0.09)
     - C24:0/C22:0, 1.71 + 0.23 (normal: 0.84 + 0.10)
     - C26:0/C22:0, 0.07 + 0.03 (normal: 0.01 + 0.004)
   - Patient has a confirmed *ABCD1* mutation detected by molecular genetic testing; **AND**
3. Patient has active central nervous system (CNS) disease established by central radiographic review of brain magnetic resonance imaging (MRI) demonstrating:
   - Loes score between 0.5–9 (inclusive) on the 34-point scale; **AND**
   - Gadolinium enhancement on MRI of demyelinating lesions; **AND**
4. Patient has a neurological function score (NFS) less than or equal to 1 (asymptomatic or mildly symptomatic disease); **AND**
5. Patient has been screened for hepatitis B virus (HBV), hepatitis C virus (HCV), human T-lymphotrophic virus 1 and 2 (HTLV-1/HTLV-2), and human immunodeficiency virus 1 and 2 (HIV-1/HIV-2) in accordance with clinical guidelines prior to collection of cells (leukapheresis); **AND**
6. Patient does not have an active infection, including clinically important localized infections; **AND**

7. Prophylaxis for infection will be followed according to standard institutional guidelines; **AND**
8. Vaccinations will not be administered within the six weeks prior to the start of therapy and will not be administered concurrently while on therapy **and** the patient is up to date with all age-appropriate vaccinations, in accordance with current vaccination guidelines, prior to initiating therapy; **AND**
9. Patient will receive periodic life-long monitoring for hematological malignancies; **AND**
10. Patient will avoid concomitant therapy with anti-retroviral medications for at least one month prior to initiating medications for stem cell mobilization and for the expected duration for elimination of the medications, and until all cycles of apheresis are completed; **AND**
11. Patient does not have head trauma induced disease; **AND**
12. Therapy will not be used to prevent the development of or treat adrenal insufficiency due to adrenoleukodystrophy; **AND**
13. Patient does not have a history of hematopoietic stem cell transplant; **AND**
14. Patient does not have a known or available human leukocyte antigen (HLA)-matched willing family donor.

Limitation

A single dose containing a minimum of 5×10^6 CD34+ cells/kg of body weight in 1 or more infusion bag(s) per lifetime.

Criteria for Denial

Above criteria are not met.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	12/13/2022
Commissioner Designee	Approval	01/26/2023
DUR Board	Revision	05/07/2024
Commissioner Designee	Approval	06/10/2024