MVP Health Care Medical Policy

Medicare Part B: Skysona

Type of Policy:	**Drug/Medical Therapy**
Prior Approval Date:	**12/01/2024**
Approval Date:	**12/01/2025**
Effective Date:	**02/01/2026**
Related Policies:	**N/A**

Refer to the MVP Medicare website for the Medicare Part D formulary and Part D policies.

Refer to relevant CMS LCDs/NCDs/Policy Articles for most up to date Medicare Part B guidance if available.

Drugs Requiring Prior Authorization under the medical benefit

J3590 Skysona (elivaldogene autotemcel)

Overview/Summary of Evidence

Skysona is one time an autologous hematopoietic stem cell (HSC)-based gene therapy that is prepared from the members HSCs through apheresis procedure. Skysona is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD) without an available human leukocyte antigen (HLA)-matched donor for allogeneic hematopoietic stem cell transplant. Early, active CALD refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9.

CALD is a rare, progressive, neurodegenerative disease that primarily affects young boys and causes irreversible, devastating neurologic decline, including major functional

disabilities such as loss of communication, cortical blindness, requirement for tube feeding, total incontinence, wheelchair dependence, or complete loss of voluntary movement. Nearly half of members who do not receive treatment die within five years of symptom onset.

Skysona has been approved under accelerated approval based on a 24-month Major Functional Disability (MFD)-free survival. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Indications/Criteria

Skysona may be considered for coverage when the following criteria are met:

- Documented diagnosis of early active cerebral adrenoleukodystrophy (CALD) and documentation of the following:
    - No human leukocyte antigen (HLA)-matched donor for allogeneic hematopoietic stem cell transplant available
    - Neurologic function score (NFS)≤ 1
    - Current magnetic brain resonance imaging (MRI) with use of Gadolinium Enhancement (GdE +) demonstrating demyelinating lesions
    - Loes scores of 0.5-9 based on assessment of brain MRI
    - Elevated very long chain fatty acid (VLCFA) confirmed by laboratory documentation
    - Confirmed mutations on the ABCD1 gene (not full deletion of the gene)
- If applicable, provider attestation confirming that anti-retroviral therapy will stop 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 complete. Anti-retroviral medications may interfere with manufacturing of the apheresed cells.
- Member's biological sex is male
- Member is 4 years to 17 years of age

- Documentation that the member has been screened for the following: hepatitis B virus (HBV), hepatitis C virus (HCV), human immunodeficiency virus 1 and 2 (HIV-1, HIV-2), human T-lymphotropic virus 1 and 2 (HTLV-1, HTLV-2).
    - Laboratory documentation indicates that the member is negative for HIV-1, HIV-2, HTLV-1, and HTLV-2
- Confirmation that member has not received any vaccinations at least 6 weeks prior to the start of myeloablative conditioning
- Confirmation that member has been assessed for hepatic and renal impairment to ensure hematopoietic stem cell (HSC) transplantation is appropriate
    - Can be confirmed by recent bloodwork (review LFTs, total bilirubin, eGFR)
- Provider attestation that full myeloablative and lymphodepleting conditioning would be administered prior to infusion of Skysona.
- Provider attestation that member will be monitored closely for evidence of malignancy through complete blood counts at least every 3 months and through assessments for evidence for clonal expansion or predominance at least twice in the first year after Skysona administration and annually thereafter
- Skysona must be administered at a qualified treatment center. Please see link for treatment centers below: SKYSONA™ (elivaldogene autotemcel) Qualified Treatment Center Locator

Skysona will be approved as **a one-time dose** and will not need to be continued for maintenance. Coverage is contingent on eligibility at the time of infusion.

Exclusions

The use of Skysona will not be covered for the following situations:

- More than one treatment per lifetime
- Age, dose, frequency of dosing, and/or duration of therapy outside of FDA approved package labeling
- For treatment of CALD secondary to head trauma
- For treatment or prevention of adrenal insufficiency
- Requests for replacement due to lost or damaged product will not be covered
- Active infection

- Member is positive for HIV-1, HIV-2, HTLV-1, and /or HTLV-2
- Full deletion of the ABCD1 gene (may result in rapid loss of efficacy due to immune response)

References

1. Skysona (elivaldogene autotemcel) suspension for intravenous infusion. Prescribing Information. Somerville, MA: Bluebird Bio, Inc; 2022. Revised August 2025.
2. Clinical Pharmacology. Skysona. Accessed October 3, 2022.
3. X-linked adrenoleukodystrophy - About the Disease - Genetic and Rare Diseases Information Center (nih.gov)
4. Clinical Pharmacology. Skysona. Accessed November 1, 2023.
5. Micromedex Healthcare Series. Skysona. Accessed November 1, 2023.
6. Eichler F, Duncan C, Musolino PL, et al. Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. The New England journal of medicine. 2017;377(17):1630-1638. doi:https://doi.org/10.1056/NEJMoa1700554 Accessed November 1, 2023.