

PHARMACY POLICY STATEMENT

Mississippi Medicaid

DRUG NAME	Skysona (elivaldogene autotemcel)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Skysona (also known as eli-cel) was granted accelerated approval by the FDA in 2022 and 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.

Skysona is a one-time autologous hematopoietic stem cell-based gene therapy in which patient stem cells are removed and modified to add functional copies of the *ABCD1* gene and then transplanted back into the patient. Skysona has a boxed warning for hematologic malignancy.

CALD, the most severe type of ALD, is a rare genetic disorder that mostly affects males, caused by mutations in *ABCD1* on the X chromosome. The *ABCD1* gene encodes the protein ALDP which is involved in the transport of very long-chain fatty acids (VLCFA). When ALDP is defective, VLCFA accumulate in plasma and tissue, including the white matter of the brain. Disease manifests as inflammatory cerebral demyelination. The result is progressive, irreversible neurologic decline leading to early death unless there is eligibility to undergo hematopoietic stem cell transplant (HSCT) early in the disease process.

Skysona (elivaldogene autotemcel) will be considered for coverage when the following criteria are met:

Cerebral Adrenoleukodystrophy (CALD)

For **initial** authorization:

1. Member is a male, 4 to 17 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist, endocrinologist, or hematologist/oncologist; AND
3. Member has a diagnosis of CALD confirmed by **both** of the following:
 - a) Elevated levels of very long chain fatty acids (VLCFA)
 - b) Genetic testing that shows mutation of the *ABCD1* gene; AND
4. Early, active disease defined by **all** the following:
 - a) Asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1)
 - b) Gadolinium enhancement on brain magnetic resonance imaging (MRI) of demyelinating lesions
 - c) Loes score of 0.5 to 9; AND
5. Member does NOT have an available human leukocyte antigen (HLA)-matched donor for allogeneic HSCT; AND
6. Member has screened negative for hepatitis B (HBV), hepatitis C (HCV), human immunodeficiency virus 1 & 2 (HIV-1/HIV-2) and Human T-lymphotropic virus 1 & 2 (HTLV-1/HTLV-2); AND
7. Complete blood count (CBC) shows ALL of the following:
 - a) Peripheral blood absolute neutrophil count ≥ 1,500 cells/mm³
 - b) Platelet count ≥ 100,000 cells/mm³

- c) Hemoglobin ≥ 10 g/dL; AND
- 8. Member does NOT have an immediate family member with a known or suspected Familial Cancer Syndrome; AND
- 9. Member has NOT had prior allogeneic transplant or gene therapy.
- 10. **Dosage allowed/Quantity limit:** Single dose in 1 or 2 infusion bags, with a minimum dose 5.0×10^6 CD34+ cells/kg administered via IV infusion (QL: two, 20 mL bags)

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization:**

- 1. Skysona is not indicated for continuous use. It is a one-time therapy.

TrueCare considers Skysona (elivaldogene autotemcel) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
10/04/2022	New policy for Skysona created.
10/21/2024	Updated references.
10/17/2025	Updated references. Changed “matched sibling donor” to “an available human leukocyte antigen (HLA)-matched donor” to match updated indication wording. Added restriction for family history of a familial cancer syndrome and added specific qualifying values to CBC, from clinical trial, due to increased reporting of hematologic malignancy.

References:

1. Skysona [prescribing information]. bluebird bio, Inc.; 2025.
2. Eichler F, Duncan C, Musolino PL, et al. Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. *N Engl J Med.* 2017;377(17):1630-1638. doi:10.1056/NEJMoa1700554
3. Raymond GV, Moser AB, Fatemi A. X-Linked Adrenoleukodystrophy. 1999 Mar 26 [Updated 2023 Apr 6]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1315/>
4. Gupta AO, Raymond G, Pierpont EI, et al. Treatment of cerebral adrenoleukodystrophy: allogeneic transplantation and lentiviral gene therapy. *Expert Opin Biol Ther.* 2022;22(9):1151-1162. doi:10.1080/14712598.2022.2124857
5. Engelen M, van Ballegoij WJC, Mallack EJ, et al. International Recommendations for the Diagnosis and Management of Patients With Adrenoleukodystrophy: A Consensus-Based Approach. *Neurology.* 2022;99(21):940-951. doi:10.1212/WNL.0000000000201374

Effective date: 04/01/2026

Revised date: 10/17/2025