

PHARMACY POLICY STATEMENT

North Carolina Marketplace

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). 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. It is a one-time 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 a matched sibling donor for 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) has been done to monitor for hematologic malignancy; AND
8. Member has NOT had prior allogeneic transplant or gene therapy.
9. **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.

CareSource 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.

References:

1. Skysona [prescribing information]. bluebird bio, Inc.; 2024.
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. Engelen M, Kemp S, de Visser M, et al. X-linked adrenoleukodystrophy (X-ALD): clinical presentation and guidelines for diagnosis, follow-up and management. *Orphanet J Rare Dis*. 2012;7:51. Published 2012 Aug 13. doi:10.1186/1750-1172-7-51
5. Turk BR, Theda C, Fatemi A, Moser AB. X-linked adrenoleukodystrophy: Pathology, pathophysiology, diagnostic testing, newborn screening and therapies. *Int J Dev Neurosci*. 2020;80(1):52-72. doi:10.1002/jdn.10003
6. 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

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