

RX.PA.090.MPC Skysona (elivaldogene autotemcel)

PURPOSE

The purpose of this policy is to define the prior authorization process for Skysona (elivaldogene autotemcel intravenous infusion).

Skysona, an autologous hematopoietic stem cell-based gene therapy, is indicated to slow the progression of neurologic dysfunction in boys 4 to 17 years of age with early, active cerebral adrenoleukodystrophy.

PROCEDURE

A. Initial Authorization Criteria:

**Please note that the provider must submit clinical documentation (chart notes, laboratory results and any other clinical support).*

Must meet all of the criteria listed under the respective diagnosis:

1. Cerebral adrenoleukodystrophy. Approve one-time (lifetime) dose if the patient meets the following:

- Patient is a male; AND
- Patient is ≥ 4 and < 18 years of age; AND
- Patient has early, active cerebral adrenoleukodystrophy as demonstrated by meeting the following:
 - Patient has a neurologic function score ≤ 1 AND
 - Patient has gadolinium enhancement on brain magnetic resonance imaging (MRI); AND
 - Patient has a Loes score between 0.5 and 9 AND
- Patient has a confirmed mutation in the adenosine triphosphate binding cassette, sub family D member 1 (*ABCD1*) gene ; AND
- Patient has elevated very long chain fatty acid levels according to the standard reference values of the laboratory ; AND
- Patient does not currently have an active bacterial, viral, fungal, or parasitic infection as determined by the prescribing physician; AND
- Patient does not have any of the following:
 - Prior or current hematologic malignancy or myeloproliferative disorder; AND
 - Familial cancer syndrome or a history of such in his immediate family; AND
- Member is clinically stable and would be considered a candidate for allogeneic hematopoietic stem cell transplantation (HSCT), but ineligible due to the absence

of an appropriate HLA-matched family donor or any other condition(s) that the provider attests which makes the member ineligible for HSCT; AND

- Patient has not received prior allogeneic HSCT, Skysona or any other gene therapy previously; AND
- Provider attests that there are no clinically significant hematologic, renal, hepatic, or infectious conditions; AND
- Patient will have treatment administered at a Skysona Qualified Treatment Center (QTC) and follow all components of the Skysona protocol: AND
- Medication is prescribed by a hematologist, a neurologist, and/or a stem cell transplant specialist; AND
- The single dose is given intravenously which contains a minimum of 5.0×10^6 CD34+ cells/kg of body weight in which body weight is based on patient weight prior to first apheresis.

B. Must be prescribed at a dose within the manufacturer’s dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Skysona will be considered investigational or experimental for any other use and will not be covered.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	1 dose
Reauthorization	N/A

If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.

APPLICABLE CODES:	
CODE	DESCRIPTION
J3590	Unclassified biologics

REFERENCES

1. Skysona® intravenous infusion [prescribing information]. Cambridge, MA: Bluebird Bio; September 2022.
2. X-linked cerebral adrenoleukodystrophy. National Institute of Health: Genetic and Rare Disease Information Center Website. Available at: <https://rarediseases.info.nih.gov/diseases/9412/x-linked-cerebral-adrenoleukodystrophy>. Created November 8, 2021. Accessed on November 1, 2022.
3. Alsaleem M, Saadeh L. Adrenoleukodystrophy. [Updated 2021 Nov 25]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2022 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK562328/>. Accessed on November 1, 2022.
4. Keam SJ. Elivaldogene autotemcel: first approval. *Mol Diagn Ther.* 2021;25(6):803-809.

5. Eichler F, Dunvan C, Musolino PL, et al. Hematopoietic stem-cell gene therapy for cerebral adrenoleukodystrophy. *N Engl J Med.* 2017;377(17):1630-1638.
6. Data on File for Skysona. Bluebird Bio. Received November 1, 2022.
7. Engelen M, Van Ballegoij WJ, Mallack EJ, et al. International recommendations for the diagnosis and management of patients with adrenoleukodystrophy: a consensus-based approach. *Neurology.* 2022 Sep 29. [Online ahead of print].

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
<i>Annual Review</i>	<i>02/2026</i>
<i>Annual Review</i>	<i>02/2025</i>
<i>Annual Review</i>	<i>02/2024</i>
<i>New Policy</i>	<i>06/2023</i>