Skysona (elivaldogene autotemcel)

Humana

Medicaid Medical Coverage Policy

Original Effective Date: 01/01/2025
Effective Date: 01/01/2025
Review Date: 08/19/2024
Policy Number: HUM-2210-000
Line of Business: Medicaid
State(s): SC

Table of Contents

Description

Adrenoleukodystrophy (ALD) is a rare X-linked metabolic disorder resulting from *ABCD1* gene mutations with an estimated incidence of 1/20,000 to 1/30,000 males. The most severe, cerebral form (CALD) develops in 35% of males less than or equal to 10 years of age with ALD. CALD, is a rare neurologic disease caused by mutations in the *ABCD1* gene that leads to a buildup of very long chain fatty acids (VLCFA) causing inflammation and damage in the brain. If not treated in a timely manner, inflammatory cerebral demyelination in CALD leads to loss of neurological and cognitive function and death, typically in early childhood. Neurologic progression of CALD may manifest as major functional disabilities, including loss of communication, movement, and mobility; blindness; tube feeding dependence; and incontinence.[1]

Allogeneic hematopoietic stem cell transplantation (HSCT) may delay the progression of childhood CALD. However, this treatment has several limitations. It is only indicated for patients in the early stages of the disease who show evidence of central nervous system involvement but no neurological symptoms. The most successful outcomes to date are reported in those patients who received cells from human leukocyte antigen (HLA)-identical, related donors unaffected with the disorder. In addition, allogeneic HCT is a major procedure that carries significant risks, including infection and graft-versus-host disease. There is an unmet need for treatments that arrest progression of CALD; **Skysona (elivaldogene autotemcel)** is a lentiviral-base gene therapy developed to address this need.[5]

Skysona is a lentiviral vector-based gene therapy intended as an early treatment to prevent demyelination and disease progression in children with CALD. This gene therapy uses a lentiviral vector to transduce an individual's HSCs to produce functional human adrenoleukodystrophy protein (ALDP) in patients with CALD. Skysona is intended for use in individuals without HLA-matched sibling donors and as an alternative treatment for those with matched donors. It is intended as a one-time treatment to permanently express functional ALD protein in individuals' blood cells.[3] Skysona does not prevent the development of or treat adrenal insufficiency due to adrenoleukodystrophy.[10]

Requests for Skysona (elivaldogene autotemcel) ***require review by a medical director*****.**

Coverage Determination

Refer all requests or questions regarding Skysona (elivaldogene autotemcel) to the Corporate Transplant Department.

Phone	Fax	Email
1-866-421-5663	502-508-9300	transplant@humana.com

Humana members may be eligible under the Plan for **Skysona (elivaldogene autotemcel)** when the following criteria are met:

- Absence of limitations; **AND**
- Individual is a male between 4 through 17 years of age with a diagnosis of early, active CALD (asymptomatic or mildly symptomatic) confirmed by all of the following[10]:
  - Elevated VLCFA; **AND**
  - Gadolinium enhancement on brain magnetic resonance imaging (MRI) with Loes score of 0.5 to 9 on the 34-point scale; **AND**
  - Neurologic function score (NFS) less than or equal to 1; **AND**
- Individual will receive 1 dose per lifetime; **AND**
- Individual will receive Skysona at a certified treatment center

Coverage Limitations

Humana members may **NOT** be eligible under the Plan for **Skysona (elivaldogene autotemcel)** for any indications other than those listed above including, but may not be limited to:

- CALD secondary to head trauma[10]; **OR**

- Clinically significant and active bacterial, fungal, parasitic, severe concomitant diseases or viral infection including hepatitis B or C (HBV, HCV), or human immunodeficiency virus (HIV)[10]; **OR**

- Prior allogeneic HSCT or gene therapy

This is considered experimental/investigational as it is not identified as widely used and generally accepted for any other proposed use as reported in nationally recognized peer-reviewed medical literature published in the English language.

Coding Information

Any codes listed on this policy are for informational purposes only. Do not rely on the accuracy and inclusion of specific codes. Inclusion of a code does not guarantee coverage and/or reimbursement for a service or procedure.

CPT® Code(s)	Description	Comments
No code(s) identified		
CPT® Category III Code(s)	**Description**	**Comments**
No code(s) identified		
HCPCS Code(s)	**Description**	**Comments**
C9399	Unclassified drugs or biologicals	
J3490	Unclassified drugs	
J3590	Unclassified biologics	

References

1. Chiesa R, Boelens J, Duncan C, et al. Variables affecting outcomes after allogeneic hematopoietic stem cell transplant for cerebral adrenoleukodystrophy. *Blood Adv*. 2022;6(5):1512-1524.

2. ClinicalKey. Drug Monograph. Elivaldogene autotemcel. https://www.clinicalkey.com. Updated December 2, 2023.

3. ECRI Institute. Genetic Test Assessment. Skysona (elivaldogene autotemcel) gene therapy (Bluebird Bio, Inc.) for early cerebral adrenoleukodystrophy. https://www.ecri.org. Published April 17, 2023.

4. Eichler F, Duncan C, Musolino P, et al. Hematopoietic stem-cell gene therapy for cerebral adrenoleukodystrophy. *N Engl J Med*. 2017;377(17):1630-1638.

5. Hayes, Inc. Emerging Technology Report. Skysona (elivaldogene autotemcel) for cerebral adrenoleukodystrophy. https://evidence.hayesinc.com. Published September 19, 2022.

6. IBM Micromedex. Elivaldogene autotemcel (Skysona). https://www.micromedexsolutions.com Updated May 22, 2023.

7. Kumar S, Sait H, Polipalli S, et al. Loes score: clinical and radiological profile of 22 patients of X-linked adrenoleukodystrophy: case series from a single center. *Indian J Radiol Imaging*. 2021;31(2):383-390.

8. Miller W, Mantovani L, Muzic J, et al. Intensity of MRI gadolinium enhancement in cerebral adrenoleukodystrophy: a biomarker for inflammation and predictor of outcome following transplantation in higher risk patients. *Amer J Neuroradiology.* 2015;37(2).500-506.

9. UpToDate, Inc. Management and prognosis of X-linked adrenoleukodystrophy. https://www.uptodate.com. Updated November 2023.

10. UpToDate, Inc. Overview of gene therapy, gene editing, and gene silencing. https://www.uptodate.com. Updated November 22, 2023.

11. US Food & Drug Administration (FDA). Full prescribing information: Skysona (elivaldogene autotemcel). https://www.fda.gov. Published September 2022.

Appendix

Appendix A
Cerebral Adrenoleukodystrophy Neurologic Function Score (NFS)

Gross clinical neurologic status	Score
Hearing/auditory processing problems	1
Aphasia/apraxia	1
Loss of communication	3
Vision impairment/ fields cut	1
Cortical blindness	2
Swallowing difficulty or other central nervous system dysfunction	2
Tube feeding	2
Running difficulties/hyperreflexia	1
Walking difficulties/ spasticity/ spastic gait (no assistance)	1
Spastic gait (needs assistance)	2
Wheelchair required	2
No voluntary movement	3
Episodes of urinary or fecal incontinency	1
Total urinary or fecal incontinency	2
Nonfebrile seizures	1
Possible Total	**25**

Appendix B
MRI Severity Scale Scoring (Loes Scoring)

<table>
<tr><th colspan="2">Each region is given a score of 0 for normal, 0.5 for unilateral involvement, and 1 for bilateral involvement or atrophy. The maximum score is 34.</th></tr>
<tr><td>Parieto-occipital white matter (maximum 4)</td><td rowspan="2">Basal ganglia (maximum 1)</td></tr>
<tr><td>Anterior temporal white matter (maximum 4)</td></tr>
<tr><td>Frontal white matter (maximum 4)
Periventricular
Central
Subcortical
Local atrophy</td><td>Visual pathway (maximum 4)
Optic radiation
Meyer’s loop
Lateral geniculate body
Optic tract</td></tr>
<tr><td>Corpus callosum (maximum 5)
Splenium
Genu
Body
Splenium atrophy
Genu atrophy</td><td>Auditory pathway (maximum 4)
Medial geniculate body
Brachium of inferior colliculus
Lateral lemniscus
Pons</td></tr>
<tr><td rowspan="2">Global atrophy (maximum 4)
Mild
Moderate
Severe
Brainstem</td><td>Cerebellum (maximum 2)
White matter
Atrophy</td></tr>
<tr><td>Projection fibers (maximum 2)
Internal capsule
Brain stem</td></tr>
</table>

Change Summary

01/01/2025 New Policy.