Skysona (elivaldogene autotemcel)

Humana

Medicaid Medical Coverage Policy

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State(s): SC

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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 10 years of age or younger 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. Without timely treatment, inflammatory cerebral demyelination (damaged or destroyed protective covering around nerve fibers) 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) has the potential to slow the progression of childhood CALD. However, its use is limited in an individual in the early stages of the disease who show signs of central nervous system involvement but have not yet developed noticeable neurological symptoms. To date, the most favorable outcomes to date are reported in an individual who received stem cells from human leukocyte antigen (HLA)-identical, related donors unaffected with the disorder. However, allogeneic HCT is a major procedure that carries significant risks, including infection and graft-versus-host disease. There remains a need for treatments that can effectively slow or halt the progression of CALD. **Skysona (elivaldogene autotemcel)** is a lentiviral-based gene therapy that has been developed as a potential approach to address this need.[5]

Skysona (elivaldogene autotemcel) is intended as an early treatment to prevent demyelination and disease progression in children with CALD. This gene therapy uses a lentiviral vector (specially engineered virus) to modify an individual's HSCs, enabling them to produce functional human adrenoleukodystrophy protein (ALDP) in an individual with CALD. Skysona is designed as a one-time gene therapy for use in individuals without HLA-matched sibling donors and as an alternative treatment for those with matched donors. The therapy aims to permanently express functional ALD protein in an individual's blood cells.[3] Skysona does not prevent the development of or treat adrenal insufficiency due to adrenoleukodystrophy.[14]

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)** *single, one-time lifetime dose* when the following criteria are met[14]:

- 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:
  - Gadolinium enhancement on brain MRI with Loes score of 0.5 to 9; **AND**
  - Neurologic function score (NFS) less than or equal to 1; **AND**
- Individual is without an available HLA-matched donor for allogeneic HSCT; **AND**
- Individual has been screened for the following: HBV, HCV, HIV-1/HIV-2 and HTLV-1/HTLV-2

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[14]:

- CALD secondary to head trauma; **OR**

- Hepatic or renal impairment; **OR**
- Individual has desire to become pregnant/reproduce OR unwilling to use effective contraception; **OR**
- Individual is pregnant or breastfeeding; **OR**
- Individual with a full *ABCD1* gene deletion

A review of the current medical literature shows that the **evidence is insufficient** to determine that this service is standard medical treatment. There is an absence of current, widely-used treatment guidelines or acceptable clinical literature examining benefit and long-term clinical outcomes establishing the value of this service in clinical management.

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	

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13. US Food & Drug Administration (FDA). FDA investigating serious risk of hematologic malignancy following Skysona (elivaldogene autotemcel). https://fda.gov. Published November 27, 2024.

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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)

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.	
Parieto-occipital white matter (maximum 4)	Basal ganglia (maximum 1)
Anterior temporal white matter (maximum 4)	
Frontal white matter (maximum 4) • Periventricular • Central • Subcortical • Local atrophy	Visual pathway (maximum 4) • Optic radiation • Meyer's loop • Lateral geniculate body • Optic tract
Corpus callosum (maximum 5) • Splenium • Genu • Body • Splenium atrophy • Genu atrophy	Auditory pathway (maximum 4) • Medial geniculate body • Brachium of inferior colliculus • Lateral lemniscus • Pons
Global atrophy (maximum 4) • Mild	Cerebellum (maximum 2) • White matter

• Moderate • Severe • Brainstem	• Atrophy
	Projection fibers (maximum 2) • Internal capsule • Brain stem

Change Summary

01/01/2025 New Policy.
10/07/2025 Annual Review, Coverage Change.