

# SmartPA Criteria Proposal

<b>Drug/Drug Class:</b>	Skysona Clinical Edit
<b>First Implementation Date:</b>	TBD
<b>Proposed Date:</b>	December 15, 2022
<b>Prepared for:</b>	MO HealthNet
<b>Prepared by:</b>	MO HealthNet/Conduent
<b>Criteria Status:</b>	<input type="checkbox"/> Existing Criteria <input type="checkbox"/> Revision of Existing Criteria <input checked="" type="checkbox"/> New Criteria

## Executive Summary

**Purpose:** Ensure appropriate utilization and control of Skysona® (elivaldogene autotemcel)

**Why Issue Selected:** Skysona® (elivaldogene autotemcel) is the first FDA-approved therapy shown to slow the progression of cerebral adrenoleukodystrophy (CALD). Adrenoleukodystrophy (ALD), also known as X-linked adrenoleukodystrophy, is a rare genetic disorder that affects the white matter of the central nervous system (CNS) and the adrenal cortex. ALD is broken down into subtypes including adrenomyeloneuropathy (AMN), adult cerebral ALD, childhood cerebral ALD (cCALD, more commonly known as CALD), and Addison's-only ALD. CALD is the most severe form and typically presents between three and ten years of age. Patients will develop normally and then start to show a loss of previously acquired skills. Many exhibit behavioral problems including attention deficit disorder and learning disabilities. Neurologic deterioration that includes increasing cognitive and behavioral abnormalities, blindness, and the development of quadriparesis may occur. Skysona is intended to be a one-time gene therapy and is designed to treat the underlying cause of CALD.

Due to the high cost, possible adverse events, and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of Skysona.

Program-Specific Information:	Drug	Cost per unit (WAC)
	SKYSONA INFUSION BAG-CASSETTE	\$3,000,000.00

**Type of Criteria:**  Increased risk of ADE  Preferred Drug List  
 Appropriate Indications  Clinical Edit

**Data Sources:**  Only Administrative Databases  Databases + Prescriber-Supplied

## Setting & Population

- Drug class for review: Skysona® (elivaldogene autotemcel)
- Age range: All appropriate MO HealthNet participants aged 4 to 17 years

## Approval Criteria

- Documented diagnosis of early, active CALD including:
  - Elevated very long chain fatty acids (VLCFA) values **AND**
  - Genetic testing confirmed pathogenic variant(s) in *ABCD1* gene **AND**
  - Active CNS disease established by central radiographic review of brain MRI demonstrating:
    - Loes score between 0.5 and 9 (inclusive) on the 34-point scale **AND**
    - Gadolinium enhancement on MRI of demyelinating lesions **AND**
  - Neurologic function score (NFS)  $\leq 1$  **AND**
- Participant aged 4 to 17 years **AND**
- Participant must be evaluated for availability of matched donors for hematopoietic stem cell transplantation (HSCT)

## Denial Criteria

- Therapy will be denied if all approval criteria are not met
- Previous therapy with Skysona at any time
- History of HSCT
- Known and available HLA-matched family donor

## Required Documentation

Laboratory Results:  
MedWatch Form:

X

Progress Notes:  
Other:

X
X

## Disposition of Edit

Denial: Exception code "0682" (Clinical Edit)  
Rule Type: CE

## Default Approval Period

3 months

## References

- Skysona® (elivaldogene autotemcel) [package insert]. Somerville, MA: bluebird bio, Inc.; September 2022.
- IPD Analytics: New Drug Review: Skysona (elivaldogene autotemcel). Accessed 8 October 2022.
- Wanders R, Eichler F. X-linked adrenoleukodystrophy and adrenomyeloneuropathy. UpToDate. Updated 17 February 2022. Accessed 8 October 2022.

*SmartPA Clinical Proposal Form*

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