SmartPA Criteria Proposal

Drug/Drug Class:	Skysona Clinical Edit
First Implementation Date:	TBD
Proposed Date:	December 15, 2022
Prepared for:	MO HealthNet
Prepared by:	MO HealthNet/Conduent
Criteria Status:	☐Existing Criteria ☐Revision of Existing Criteria ☒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**
☒ **Appropriate Indications**
☐ **Preferred Drug List**
☒ **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 variants 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) ≤ 1 **AND**
- Participant aged 4 to 17 years

Denial Criteria

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

Required Documentation

Laboratory Results:	X	Progress Notes:	X
MedWatch Form:		Other:	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.