Medical Policy:
Nulibry™ (fosdenopterin)

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.338	February 1, 2024	June 9, 2021

Definitions

Nulibry, a cyclic pyranopterin monophosphate (cPMP), is indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A. MoCD is a rare, life-threatening, autosomal-recessive disorder characterized by the deficiency of three molybdenum-dependent enzymes: sulfite oxidase (SOX), xanthine dehydrogenase, and aldehyde oxidase. Patients with MoCD Type A have mutations in the *MOCS1* gene leading to deficiency of the intermediate substrate, cPMP. Substrate replacement therapy with Nulibry provides an exogenous source of cPMP, which is converted to molybdopterin. Molybdopterin is then converted to molybdenum cofactor, which is needed for the activation of molybdenum-dependent enzymes, including SOX, an enzyme that reduces levels of neurotoxic sulfites.

Length of Authorization

Coverage will be provided for 1 year and may be renewed.

Dosing Limits [Medical Benefit]

Approve up to 0.9 mg/kg given by intravenous infusion once daily.

Max Units (per dose and over time) [HCPCS Unit]:

95 mg daily

Guideline

I. INITIAL APPROVAL CRITERIA

1. **Molybdenum Cofactor Deficiency (MoCD) Type A:**
   Coverage will be provided if the patient meets the following criteria (A, B, and C):
   A. Patient has genetic testing confirmation of a mutation in the *MOCS1* gene; **AND**
   B. According to the prescriber, based on the current condition, the patient is expected to derive benefit with Nulibry and the disease state is NOT considered to be too advanced; **AND**
   C. The medication is prescribed by or in consultation with a pediatrician, geneticist, or a physician who specializes in molybdenum cofactor deficiency (MoCD) Type A.

II. Renewal Criteria

1. **Molybdenum Cofactor Deficiency (MoCD) Type A** (A, B, and C)**:**
   Coverage can be renewed based on the following criteria:
   A. Patient continues to meet the above criteria and dosing; **AND**
   B. There is significant clinical improvement of the disease as determined by the prescribing physician; **AND**
   C. There is absence of unacceptable toxicity from the drug.

Dosing/Administration

Age less than 1 year (Pre-Term neonates - gestational age < 37 weeks:
Initial dosage: 0.4 mg/kg once daily − Dosage at 1 month: 0.7 mg/kg once daily − Dosage at 3 months: 0.9 mg/kg once daily

Age less than 1 year (Full-Term neonates - gestational age ≥37 weeks)
Initial dosage: 0.55 mg/kg once daily − Dosage at 1 month: 0.75 mg/kg once daily − Dosage at 3 months: 0.9 mg/kg once daily

Age 1 year and older: The recommended dosage is 0.9 mg/kg administered as an IV infusion once daily, based on actual body weight.

Applicable Procedure Codes

Code	Description
C9399	NULIBRY 9.5MG Solution Reconstituted, Unclassified drugs or biologicals
J3490	NULIBRY 9.5MG Solution Reconstituted, Unclassified drugs

Applicable NDCs

Code	Description
73129-0001-xx	NULIBRY Single-Dose Vial for Intravenous Infusion: 9.5 MG/vial

ICD-10 Diagnoses

Code	Description

E61.5	Molybdenum deficiency
E72.19	Other disorders of sulfur-bearing amino-acid metabolism

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	2/1/2024	Annual Review: no criteria changes
EmblemHealth & ConnectiCare	5/30/2023	Annual Review: no criteria changes
EmblemHealth & ConnectiCare	09/13/2022	Transferred policy to new template.
EmblemHealth & ConnectiCare	9/21/2021	Updated diagnosis codes
EmblemHealth & ConnectiCare	6/9/2021	New Policy

References

1. Nulibry™ (fosdenopterin) for injection [package insert]. Boston, MA. Origin Biosciences, Inc. Updated March 10, 2021. Available at: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=4f67cc4e-84ed-4f4e-a5d9-6ffbfb84eddd
2. Nulibry™(fosdenopterin) for injection. IBM Micromedex® [database online]. Greenwood Village, CO. Truven Health Analytics. Available at: https://www.micromedexsolutions.com. Updated March 29, 2021. Accessed April 8, 2021.
3. Mechler K, Mountford WK, Hoffmann GF, et al. Ultra-orphan diseases: a quantitative analysis of the natural history of molybdenum cofactor deficiency. *Genet Med*. 2015 Dec;17(12):965-70