CareSource Rxinnovations

# PHARMACY POLICY STATEMENT
## Ohio Medicaid

| DRUG NAME | Nulibry (fosdenopterin) |
|---|---|
| BILLING CODE | J3490, C9399, or NDC |
| BENEFIT TYPE | Medical or Pharmacy |
| SITE OF SERVICE ALLOWED | Hospital Inpatient, Outpatient, Home |
| STATUS | Prior Authorization Required |

Nulibry (fosdenopterin) is a synthetic substrate replacement therapy indicated for the treatment of molybdenum cofactor deficiency (MoCD) Type A. MoCD Type A is an ultra-rare autosomal recessive, inborn error of metabolism that results in accumulation of a neurotoxic metabolite of sulfite which causes rapid and progressive neurological damage. MoCD type A is caused by mutations in the molybdenum cofactor synthesis 1 gene (MOCS1) and presents shortly after birth.
Nulibry is the first drug to target underlying etiology and reduce the risk of mortality. Prior to Nulibry, treatment had been strictly supportive, such as anticonvulsants for seizures.

Nulibry (fosdenopterin) will be considered for coverage when the following criteria are met:

## MOLYBDENUM COFACTOR DEFICIENCY (MoCD) TYPE A

For **initial** authorization:
1. Medication must be prescribed by or in consultation with a neonatologist, geneticist, metabolic specialist, or pediatric neurologist; AND
2. ONE of the following:
   a) Member has a diagnosis of MoCD Type A confirmed by genetic testing (must show mutation in the MOSC1 gene), OR
   b) Member has a presumptive diagnosis of MoCD Type A and genetic testing is to be immediately completed.
   NOTE: If genetic testing does not confirm the diagnosis, Nulibry must be discontinued.
   NOTE: Early presenting characteristics include seizures of unknown origin, strongly positive sulfite dipstick, etc.; AND
3. Documentation of baseline S-sulfocysteine (SSC) level.
4. **Dosage allowed/Quantity limit:**
   Less than 1 year of age: Dosing based on weight per package insert
   Age 1 year or older: 0.9 mg/kg IV once daily

***If all the above requirements are met, the medication will be approved for 6 months.***

For **reauthorization**:
1. If not provided for initial authorization, genetic test result confirming MoCD Type A must be submitted; AND
2. Chart notes must show positive clinical response such as reduced convulsions, normalized biomarkers (urinary S-sulphocysteine (SSC), xanthine, urate), improved neurological or motor function, or achievement of developmental milestones.

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

**CareSource considers Nulibry (fosdenopterin) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

| DATE | ACTION/DESCRIPTION |
|---|---|
| **6/24/2021** | New policy for Nulibry (fosdenopterin) created. |

References:


1. Nulibry (fosdenopterin) [package insert]. Charleston SC; Alcami Carolinas Corporation. Revised 2/2021
2. Molybdenum cofactor deficiency. Genetics Home Reference. Accessed June 24, 2021. https://medlineplus.gov/genetics/condition/molybdenum-cofactor-deficiency/
3. Study of ORGN001 (Formerly ALXN1101) in Neonates, Infants and Children With Molybdenum Cofactor Deficiency (MOCD) Type A. ClinicalTrials.gov Identifier: NCT02629393. Updated February 26, 2021. Accessed June 30, 2021. https://clinicaltrials.gov/ct2/show/NCT02629393?term=NCT02629393&draw=2&rank=1
4. Atwal PS, Scaglia F. Molybdenum cofactor deficiency. *Mol Genet Metab*. 2016;117(1):1-4. doi:10.1016/j.ymgme.2015.11.010
5. Schwahn BC, Van Spronsen FJ, Belaidi AA, et al. Efficacy and safety of cyclic pyranopterin monophosphate substitution in severe molybdenum cofactor deficiency type A: a prospective cohort study. *Lancet*. 2015;386(10007):1955-1963. doi:10.1016/S0140-6736(15)00124-5


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