

## PHARMACY POLICY STATEMENT

### Indiana Medicaid

|                  |                                |
|------------------|--------------------------------|
| <b>DRUG NAME</b> | <b>Nulibry (fosdenopterin)</b> |
| BENEFIT TYPE     | Medical or Pharmacy            |
| STATUS           | Prior Authorization Required   |

Nulibry, approved by the FDA in 2021, is cyclic pyranopterin monophosphate (cPMP) indicated to reduce the risk of mortality in patients with 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 (SSC) which causes rapid and progressive neurological damage that usually presents shortly after birth. Mutations in the molybdenum cofactor synthesis 1 gene (MOCS1) lead to deficient synthesis of cPMP. Nulibry provides a synthetic exogenous source of cPMP as substrate replacement therapy.

Nulibry is the first drug to target the underlying etiology of MoCD Type A 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 MOCS1 gene), OR
  - b) Member has a presumptive diagnosis of MoCD Type A with early presenting characteristics such as seizures of unknown origin, exaggerated startle response, axial hypotonia, strongly positive sulfite dipstick, etc AND genetic testing is to be immediately completed.

NOTE: If genetic testing does not confirm the diagnosis, Nulibry must be discontinued; AND
3. Documentation of baseline S-sulfocysteine (SSC) level; AND
4. Documentation of member's weight.
5. **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.   |
| 04/28/2023 | Added reference. Added more examples of presenting characteristics and clarified criterion. Added requirement for weight documentation. |
| 12/08/2025 | Updated references.   |

References:

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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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7. Schwarz G, Basel DG, Schwahn BC, et al. Increased Survival in Patients With Molybdenum Cofactor Deficiency Type A Treated With Cyclic Pyranopterin Monophosphate. *J Inherit Metab Dis*. 2025;48(2):e70000. doi:10.1002/jimd.70000
8. Schwahn BC, van Spronsen F, Misko A, et al. Consensus guidelines for the diagnosis and management of isolated sulfite oxidase deficiency and molybdenum cofactor deficiencies. *J Inherit Metab Dis*. 2024;47(4):598-623. doi:10.1002/jimd.12730

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Revised date: 12/08/2025