CVS Caremark®

|  |
| --- |
| Reference number(s) |
| 4575-A |

# Specialty Guideline Management Nulibry

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Nulibry | fosdenopterin |

## Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

### FDA-approved Indication1

Nulibry is indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A.

All other indications are considered experimental/investigational and not medically necessary.

## Documentation

Submission of the following information is necessary to initiate the prior authorization review:

### Initial requests

* Genetic testing results documenting pathogenic variant(s) in the molybdenum cofactor synthesis 1 (MOCS1) gene, where applicable.

### Continuation requests (where applicable)

* Genetic testing results documenting pathogenic variant(s) in the molybdenum cofactor synthesis 1 (MOCS1) gene.
* Chart notes or medical records documenting a benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for encephalopathy and/or seizure activity, improved or normalized uric acid, urinary S-sulfocysteine, and xanthine levels).

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

## Coverage Criteria

### Molybdenum cofactor deficiency (MoCD) Type A

Authorization 12 months may be granted when the diagnosis of MoCD Type A was confirmed by genetic testing documenting pathogenic variant(s) in the molybdenum cofactor synthesis 1 (MOCS1) gene.

Authorization of 3 months may be granted when both of the following criteria are met:

* Member has a presumed diagnosis of MoCD Type A and genetic test results are pending.
* Member has clinical signs and symptoms associated with MoCD Type A (e.g., encephalopathy, intractable seizures, developmental delay, decreased uric acid levels, elevated urinary S-sulfocysteine and/or xanthine levels).

## Continuation of Therapy

Authorization of 12 months may be granted for members with an indication listed in the coverage criteria section when one of the following is met:

* The member has received less than 12 months of therapy and has genetic testing results documenting pathogenic variant(s) in the molybdenum cofactor synthesis 1 (MOCS1) gene.
* Member has received 12 months of therapy or more and is experiencing benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for encephalopathy and/or seizure activity, improved or normalized uric acid, urinary S-sulfocysteine, and xanthine levels).

## References

1. Nulibry [package insert]. Solana Beach, CA: Sentynl Therapeutics, Inc.; October 2022.
2. Atwal PS, Scaglia F. Molybdenum cofactor deficiency. Mol Genet Metab. 2016;117(1):1-4.
3. 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: 1955-1963.
4. ClinicalTrials.gov. Study of ORGN001 (formerly ALXN1101) in neonates with molybdenum cofactor deficiency (MOCD) type A. Available at: https://clinicaltrials.gov/study/NCT02629393. Accessed: November 11, 2024.
5. ClinicalTrials.gov. Safety & efficacy study of ORGN001 (formerly ALXN1101) in pediatric patients with MoCD type A currently treated with rcPMP. Available at: https://clinicaltrials.gov/ct2/show/NCT02047461. Accessed: November 11, 2024.