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 Indication[1]

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.