Fosdenopterin (Nulibry™)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

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

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) ~~a mutation~~ in the molybdenum cofactor synthesis 1 (MOSC1) gene, where applicable.

Continuation requests (where applicable)~~:~~

- Genetic testing results documenting pathogenic variant(s) a ~~mutation~~ in the molybdenum cofactor synthesis 1 (MOSC1) gene.
- Chart notes or medical records documenting a benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for encephalopathy, 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 ~~FOR INITIAL APPROVAL~~

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) ~~a mutation~~ in the molybdenum cofactor synthesis 1 (MOSC1) 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 ~~III~~ when one of the following is met:

- The member has received less than 12 months of therapy and has genetic testing results documenting a pathogenic variant(s) ~~mutation~~ in the molybdenum cofactor synthesis 1 (MOSC1*)* 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).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Nulibry [package insert]. Solana Beach, CA ~~Boston, MA~~: Sentynl Therapeutics ~~Origin Biosciences~~, 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/ct2/show/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.

EFFECTIVE DATE

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