Reference number(s)
4582-A

STANDARD MEDICARE PART B MANAGEMENT

NULIBRY (fosdenopterin)

POLICY

I. 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 cyclic pyranopterin monophosphate (cPMP) indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:

A. Initial requests: genetic testing results documenting a mutation in the molybdenum cofactor synthesis gene 1 *(MOSC1)*.

B. Continuation requests (where applicable):
1. Genetic testing results documenting a mutation in the molybdenum cofactor synthesis gene 1 *(MOSC1)*.
2. 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).

III. CRITERIA FOR INITIAL APPROVAL

Molybdenum cofactor deficiency (MoCD) Type A

A. Authorization of 12 months may be granted for treatment of MoCD Type A when the diagnosis was confirmed by genetic testing documenting a mutation in the molybdenum cofactor synthesis gene 1 *(MOSC1).*

B. Authorization of 3 months may be granted for treatment of MoCD Type A when both of the following criteria are met:
1. Member has a presumed diagnosis of MoCD Type A and genetic test results are pending.

2. 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).

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Nulibry
B. Nulibry is being used to treat an indication enumerated in Section III
C. The member meets one of the following criteria:
   1. The member has received less than 12 months of therapy and has genetic testing results documenting a mutation in the molybdenum cofactor synthesis gene 1 *(MOSC1).*
   2. The 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, seizure activity, improved or normalized uric acid, urinary S-sulfocysteine, and xanthine levels).

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:
1. The prescribing information for Nulibry.
2. The available compendium
   a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
   b. Micromedex DrugDex
   c. American Hospital Formulary Service- Drug Information (AHFS-DI)
   d. Lexi-Drugs
   e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Nulibry are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

VII. REFERENCES

1. Nulibry [package insert]. Boston, MA: Origin Biosciences, Inc.; February 2021.
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: October 27, 2022.

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: October 27, 2022.