Reference number(s)
4575-A

SPECIALTY GUIDELINE 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 are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
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 12 months may be granted when the diagnosis of MoCD Type A was confirmed by genetic testing documenting a mutation in the molybdenum cofactor synthesis gene 1 *(MOSC1).*

B. Authorization of 3 months may be granted 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

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

A. 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).*
B. 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. 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: March 2, 2021.
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: March 2, 2021.