

**Nulibry® (fosdenopterin)**  
**Effective 11/01/2021**

<b>Plan</b>	<input type="checkbox"/> MassHealth UPPL <input checked="" type="checkbox"/> Commercial/Exchange	<b>Program Type</b>	<input checked="" type="checkbox"/> Prior Authorization <input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
<b>Benefit</b>	<input type="checkbox"/> Pharmacy Benefit <input checked="" type="checkbox"/> Medical Benefit		
<b>Specialty Limitations</b>	N/A		
<b>Contact Information</b>	<b>Medical and Specialty Medications</b>		
	All Plans	Phone: 877-519-1908	Fax: 855-540-3693
<b>Exceptions</b>	<b>Non-Specialty Medications</b>		
	All Plans	Phone: 800-711-4555	Fax: 844-403-1029

### Overview

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

### Coverage Guidelines

Authorization may be reviewed for members new to the plan who are currently receiving treatment with Nulibry excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

#### OR

Authorization may be granted for members when ALL the following criteria are met, and documentation is provided:

1. Documented diagnosis of molybdenum cofactor deficiency type A
2. Documentation of ONE of the following:
  - a. Diagnosis of molybdenum cofactor deficiency type A confirmed by genetic testing
  - b. In neonates (up to 28 days after birth), diagnosis based on ONE of the following:
    - i. Prenatal genetic diagnosis
    - ii. Onset of clinical signs and symptoms consistent with molybdenum cofactor deficiency Type A (e.g., seizures, feeding difficulties, high-pitched cries, exaggerated startle reactions, increased/decreased muscle tone) within the first 28 days after birth
    - iii. Onset of laboratory signs and symptoms consistent with molybdenum cofactor deficiency Type A (e.g., elevated urinary sulfite and/or S-sulphocysteine, elevated xanthine in urine or blood, or low or absent uric acid in the urine or blood) within the first 28 days after birth

### Continuation of Therapy

Reauthorization will be granted if member meets all of the following criteria:

1. Diagnosis of molybdenum cofactor deficiency Type A confirmed by genetic testing
2. Documented therapeutic response as evidenced by ONE of the following:

- a. Improved change in molybdenum cofactor deficiency biomarkers
- b. Improved growth parameters

#### **Limitations**

1. Initial approvals will be granted for 3 months
2. Reauthorizations will be granted for 12 months

#### **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>.
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>.

#### **Review History**

09/22/2021 – Reviewed and Created for Sept P&T. Effective 11/01/2021.

