

Nulibry® (fosdenopterin)

When requesting Nulibry® (fosdenopterin intravenous infusion), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indications.

FDA-Approved Indication

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

Coverage Guidelines

Molybdenum Cofactor Deficiency (MoCD) Type A

The individual must meet **all** of the following criteria for initial authorization:

- Diagnosis was confirmed by ONE of the following:
 - Patient has genetic testing confirmation of biallelic pathogenic or likely pathogenic variants in the *MOCS1* gene; OR
 - Patient has laboratory findings suggestive of molybdenum cofactor deficiency and genetic testing is in progress;
Note: Laboratory findings include elevated urinary S-sulfocysteine, thiosulfate, xanthine, hypoxanthine, or decreased serum uric acid.
- Based on current condition, the patient is expected to derive benefit with Nulibry and the disease state is not considered to be too advanced;
- The drug is being prescribed by or in consultation with a pediatrician, geneticist, or a physician who specializes in MoCD Type A.

Approval duration (genetic testing confirmed): 12 months

Approval duration (genetic testing in progress): 1 month

Dosing Recommendations

The recommended dosage is given by intravenous infusion as follows:

Recommended Initial Dosage and Titration Schedule of Nulibry for Patients Less Than One Year of Age by Gestational Age

Titration Schedule	Preterm Neonates: Postmenstrual age (<i>gestational age plus chronological age</i>) less than 37 weeks	Term Neonates: Postmenstrual age (<i>gestational age plus chronological age</i>) 37 weeks and above

Initial dosage	0.4 mg/kg once daily	0.55 mg/kg once daily
Dosage at month 1	0.7 mg/kg once daily	0.75 mg/kg once daily
Dosage at month 3	0.9 mg/kg once daily	0.9 mg/kg once daily

For patients one year of age or older, the recommended dosage of NULIBRY is 0.9 mg/kg (based on actual body weight) administered as an intravenous infusion once daily.

References

1. Nulibry injection [prescribing information]. Boston, MA: Origin Biosciences; October 2022.
2. Mechler K, Mountford WK, Hoffmann GF, et al. Ultra-orphan diseases: a quantitative analysis of the natural history of molybdenum cofactor deficiency. *Genet Med*. 2015 Dec;17(12):965-70.
3. Misko A, Mahtani K, Abbott J, et al. Molybdenum Cofactor Deficiency. 2021 Dec 2 [Updated 2023 Feb 2]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK575630/>. Accessed on March 26, 2025