

Nulibry® (fosdenopterin) (Intravenous)

Document Number: IC-0594

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I. Length of Authorization

Coverage will be provided initially for 6 months and may be renewed annually thereafter.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 95 mg daily

III. Initial Approval Criteria ¹

Coverage is provided in the following conditions:

Universal Criteria ^{1,3}

- Will not be used in combination with other substrate replacement therapy (e.g., recombinant cyclic pyranopterin monophosphate, etc.); **AND**
- Must be prescribed by, or in consultation with, a specialist in medical genetics or pediatric neurology; **AND**

Molybdenum Cofactor Deficiency Type A (MoCD Type A) † Φ ¹⁻³

- Patient meets one of the following scenarios:
 - Patient has a diagnosis of MoCD Type A confirmed by a mutation in the *MOCS1* gene suggestive of disease as identified on molecular genetic testing; **OR**
 - Patient has biochemical features suggestive of MoCD Type A (i.e., elevated sulfites in urine, low serum uric acid, elevated urinary xanthine and hypoxanthine) and will be treated presumptively while awaiting genetic confirmation; **AND**
- Patient has a baseline value for the following:
 - Urinary s-sulfocysteine (SSC) normalized to creatinine; **AND**
 - Clinical notes regarding signs and symptoms of disease which may include, but are not limited to, seizure frequency/duration, growth, and developmental milestones

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria ¹

Coverage may be renewed based on the following criteria:

- Patient continues to meet the universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe phototoxicity, clinically significant infection, etc.; **AND**
 - Disease response compared to pre-treatment baseline as evidenced by the following:
 - Reduction in urinary SSC normalized to creatinine; **AND**
 - Stabilization or improvement in one or more signs and symptoms of disease including, but not limited to, seizure frequency/duration, growth, achievement of developmental milestones; **OR**
 - Patient initiated therapy as an inpatient based upon a presumptive diagnosis of MoCD Type A which was subsequently confirmed by genetic testing; **AND**
 - Patient is responding to therapy compared to one or more pre-treatment baseline parameters which prompted the workup for MoCD

V. Dosage/Administration ¹

| Indication | Dose |
|---|--|
| MoCD Type A | <u>Age less than 1 year (Pre-Term Neonates - Gestational Age <37 weeks)</u> |
| | – Initial dosage: 0.4 mg/kg once daily |
| | – Dosage at 1 month: 0.7 mg/kg once daily |
| | – Dosage at 3 months: 0.9 mg/kg once daily |
| | <u>Age less than 1 year (Full-Term Neonates - Gestational Age ≥37 weeks)</u> |
| | – Initial dosage: 0.55 mg/kg once daily |
| | – Dosage at 1 month: 0.75 mg/kg once daily |
| | – Dosage at 3 months: 0.9 mg/kg once daily |
| | <u>Age at least 1 year</u> |
| | – The recommended dosage is 0.9 mg/kg administered as an IV infusion once daily. |
| | <i>*Note all weights are based on Actual Body Weight (ABW)</i> |
| Nulibry is intended for intravenous administration by a healthcare provider. Nulibry may be administered at home by the patient's caregiver if deemed appropriate by a healthcare provider. | |

VI. Billing Code/Availability Information

HCPCS Code:

- J3490 – Unclassified drugs
- C9399 – Unclassified drugs or biologicals (hospital outpatient use)

NDC:

- Nulibry 9.5 mg single-dose vial as a lyophilized powder for injection: 73129-0001-xx
- Nulibry 9.5 mg single-dose vial as a lyophilized powder for injection: 42358-0295-xx

VII. References

1. Nulibry [package insert]. Solana Beach, CA; Sentyln Therapeutics, Inc.; October 2022. Accessed March 2025.
2. Origin Biosciences. A Phase 2, Multicenter, Multinational, Open-Label, Dose-Escalation Study to Evaluate the Safety and Efficacy of ORGN001 (Formerly ALXN1101) in Pediatric Patients With Molybdenum Cofactor Deficiency (MoCD) Type A Currently Treated With Recombinant Escherichia Coli-derived Cyclic Pyranopterin Monophosphate (rcPMP). Available from: <https://clinicaltrials.gov/ct2/show/NCT02047461?term=NCT02047461&draw=2&rank=1>. NLM identifier: NCT02047461. Accessed March 4, 2025.
3. Origin Biosciences. A Phase 2/3, Multicenter, Multinational, Open Label Study to Evaluate the Efficacy and Safety of ORGN001 (Formerly ALXN1101) in Neonates, Infants and Children With Molybdenum Cofactor Deficiency (MOCD) Type A. Available from: <https://clinicaltrials.gov/ct2/show/NCT02629393?term=NCT02629393&draw=2&rank=1>. NLM identifier: NCT02629393. Accessed March 4, 2025.
4. Reiss J, Hahnewald R. Molybdenum cofactor deficiency: Mutations in GPHN, MOCS1, and MOCS2. Hum Mutat. 2011 Jan;32(1):10-8.
5. Veldman A, Santamaria-Araujo JA, Sollazzo S, et al. Successful treatment of molybdenum cofactor deficiency type A with cPMP. Pediatrics. 2010 May;125(5):e1249-54. doi: 10.1542/peds.2009-2192. Epub 2010 Apr 12.

Appendix 1 – Covered Diagnosis Codes

| ICD-10 | ICD-10 Description |
|--------|---|
| E61.5 | Molybdenum deficiency |
| E72.19 | Other disorders of sulfur-bearing amino-acid metabolism |

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

| Medicare Part B Administrative Contractor (MAC) Jurisdictions | | |
|--|---|---|
| Jurisdiction | Applicable State/US Territory | Contractor |
| E (1) | CA, HI, NV, AS, GU, CNMI | Noridian Healthcare Solutions, LLC |
| F (2 & 3) | AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ | Noridian Healthcare Solutions, LLC |
| 5 | KS, NE, IA, MO | Wisconsin Physicians Service Insurance Corp (WPS) |
| 6 | MN, WI, IL | National Government Services, Inc. (NGS) |
| H (4 & 7) | LA, AR, MS, TX, OK, CO, NM | Novitas Solutions, Inc. |
| 8 | MI, IN | Wisconsin Physicians Service Insurance Corp (WPS) |
| N (9) | FL, PR, VI | First Coast Service Options, Inc. |
| J (10) | TN, GA, AL | Palmetto GBA |
| M (11) | NC, SC, WV, VA (excluding below) | Palmetto GBA |
| L (12) | DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA) | Novitas Solutions, Inc. |
| K (13 & 14) | NY, CT, MA, RI, VT, ME, NH | National Government Services, Inc. (NGS) |
| 15 | KY, OH | CGS Administrators, LLC |