Nulibry® (fosdenopterin)
(Intravenous)

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I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for 6 months (180 days).
- Renewal: Prior authorization validity may be renewed every 12 months (365 days) thereafter.

II. Dosing Limits

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

- 950 billable units daily

III. Initial Approval Criteria [1]

Prior authorization validity is provided in the following conditions:

Universal Criteria [1,3]

- Will not be used in combination with other substrate replacement therapy; **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]

- Member meets one of the following scenarios:
  - Member 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**
  - Member 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**
- Member 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]

Prior authorization validity may be renewed based on the following criteria:

- Member 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**
  - Member initiated therapy as an inpatient based upon a presumptive diagnosis of MoCD Type A which was subsequently confirmed by genetic testing; **AND**
    - Member 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. **Note all weights are based on Actual Body Weight (ABW)*
Nulibry is intended for intravenous administration by a healthcare provider. Nulibry may be administered at home by the member's caregiver if deemed appropriate by a healthcare provider.	

VI. Billing Code/Availability Information

<u>HCPCS Code:</u>

- J1809 – Injection, fosdenopterin, 0.1 mg; 1 billable unit = 0.1 mg

<u>NDC:</u>

- 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; Sentynl Therapeutics, Inc.; October 2022. Accessed February 2026.
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 February 25, 2026.
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 February 25, 2026.
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 A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime's assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	No: PA not a priority
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

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