Nulibry™ (fosdenopterin)
(Intravenous)

Document Number: IC-0594

Last Review Date: 01/05/2023
Date of Origin: 04/06/2021
Dates Reviewed: 04/2021, 01/2022, 01/2023

I. Length of Authorization

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

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Nulibry 9.5 mg vial for injection: 10 vials daily

B. 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 has a diagnosis of MoCD Type A as confirmed, by molecular genetic testing, by a mutation in the *MOCS1* gene suggestive of disease; **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:
  - Elevated 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]

Authorizations can be renewed based on the following criteria:

- Patient continues to meet 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. **Note all weights are based on Actual Body Weight (ABW)*
Nulibry is administered intravenously by a healthcare provider or at home by the patient's caregiver	

VI. Billing Code/Availability Information

<u>HCPCS Code:</u>

- 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

VII. References

1. Nulibry [package insert]. Boston, MA; Origin Biosciences, Inc.; October 2022. Accessed November 2022.
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 Marche 3, 2021.
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 3, 2021.
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, Pitt J, Gianello R, Yaplito-Lee J, Wong F, Ramsden CA, Reiss J, Cook I, Fairweather J, Schwarz G. 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)

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 Determination (NCD), Local Coverage Articles (LCAs), and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): 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, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
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

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PCHP:
Provides free aids and services to people with disabilities to communicate effectively with us, such as:

- Qualified sign language interpreters
- Written information in other formats (large print, audio, accessible electronic formats, other formats)

Provides free language services to people whose primary language is not English, such as:

- Qualified interpreters
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If you need these services, contact a Grievance Specialist.

If you believe that PCHP has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, you can file a grievance with:

Grievance Specialist
PreferredOne Community Health Plan
PO Box 59052
Minneapolis, MN 55459-0052
Phone: 1.800.940.5049 (TTY: 763.847.4013)
Fax: 763.847.4010
customerservice@preferredone.com

You can file a grievance in person or by mail, fax, or email. If you need help filing a grievance, a Grievance Specialist is available to help you.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1-800-368-1019, 800-537-7697 (TDD)

Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html.

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PreferredOne Insurance Company
PO Box 59212
Minneapolis, MN 55459-0212
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U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1-800-368-1019, 800-537-7697 (TDD)

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