

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); Ⓞ 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.
<i>*Note all weights are based on Actual Body Weight (ABW)</i>	
Nulibry is administered intravenously by a healthcare provider or at home by the patient's caregiver	

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

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 March 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, Hahnwald 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

Nondiscrimination & Language Access Policy

Aspirus Health Plan, Inc. complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability, sex, sexual orientation, or gender identity. We do not exclude people or treat them differently because of race, color, national origin, age, disability, sex, sexual orientation, or gender identity.

We will:

Provide 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)

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

- Qualified interpreters
- Information written in other languages

If you need these services, contact us at the phone number shown on the inside cover of this contract, your id card, or aspirushealthplan.com.

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

Nondiscrimination Grievance Coordinator
Aspirus Health Plan, Inc.
PO Box 1062
Minneapolis, MN 55440
Phone: 1.866.631.5404 (TTY: 711)
Fax: 763.847.4010
Email: customerservice@aspirushealthplan.com

You can file a grievance in person or by mail, fax, or email. If you need help filing a grievance, the Nondiscrimination Grievance Coordinator 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>.

Language Assistance Services

Albanian: KUJDES: Nëse flitni shqip, për ju ka në dispozicion shërbime të asistencës gjuhësore, pa pagesë. Telefononi në 1.866.631.5404 (TTY: 711).

Arabic: تنبيه: إذا كنت تتحدث اللغة العربية، فإن خدمات المساعدة اللغوية متاحة لك مجاناً. اتصل بن اعلى رقم الهاتف 1.866.631.5404 (رقم هاتف الصم والبك : 711)

French: ATTENTION : Si vous parlez français, des services d'aide linguistique vous sont proposés gratuitement. Appelezle 1.866.631.5404 (ATS : 711).

German: ACHTUNG: Wenn Sie Deutsch sprechen, stehen Ihnen kostenlos sprachliche Hilfsdienstleistungen zur Verfügung. Rufnummer: 1.866.631.5404 (TTY: 711).

Hindi: _यान द_ : य_द आप िहंदी बोलते ह_ तो आपके िलए मु_त म_ भाषा सहायता सेवाएं उपल_ध ह_। 1.866.631.5404 (TTY: 711) पर कॉल कर_।

Hmong: LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1.866.631.5404 (TTY: 711).

Korean: 주의: 한국어를 사용하시는 경우, 언어 지원 서비스를 무료로 이용하실 수 있습니다. 1.866.631.5404 (TTY: 711)번으로 전화해 주십시오.

Polish: UWAGA: Jeżeli mówisz po polsku, możesz skorzystać z bezpłatnej pomocy językowej. Zadzwoń pod numer 1.866.631.5404 (TTY: 711).

Russian: ВНИМАНИЕ: Если вы говорите на русском языке, то вам доступны бесплатные услуги перевода. Звоните 1.866.631.5404 (телетайп: 711).

Spanish: ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al 1.866.631.5404 (TTY: 711).

Tagalog: PAUNAWA: Kung nagsasalita ka ng Tagalog, maaari kang gumamit ng mga serbisyo ng tulong sa wika nangwalang bayad. Tumawag sa 1.866.631.5404 (TTY: 711)

Traditional Chinese: 注意: 如果您使用繁體中文, 您可以免費獲得語言援助服務。請致電 1.866.631.5404 (TTY:711)。

Vietnamese: CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1.866.631.5404 (TTY: 711).

Pennsylvania Dutch: Wann du Deitsch (Pennsylvania German / Dutch) schwetzsch, kannscht du mitaus Koschte ebbergricke, ass dihr helft mit die englisch Schprooch. Ruf selli Nummer uff: Call 1.866.631.5404 (TTY: 711).

Lao: ໄປ່ດຊາຍ: ຖ້າວ່າ ທ່ານເວົ້າພາສາ ລາວ, ການບໍລິການຊ່ວຍເຫຼືອດ້ານພາສາ, ໂດຍບໍ່ເສັຽຄ່າ, ແມ່ນມີພ້ອມໃຫ້ທ່ານ. ໂທ 1.866.631.5404 (TTY:711).