Medical Policy

Nulibry™ (fosdenopterin hydrobromide)	
MEDICAL POLICY NUMBER	Med_Clin_Ops_071
CURRENT VERSION EFFECTIVE DATE	January 1, 2024
APPLICABLE PRODUCT AND MARKET	*Individual Family Plan:* All Plans *Small Group*: All Plans *Medicare Advantage:* All Plans

Brand New Day/Central Health Medicare Plan develops policies and makes coverage determinations using credible scientific evidence including but not limited to MCG™ Health Guidelines, the ASAM Criteria™, and other third party sources, such as peer-reviewed medical literature generally recognized by the relevant medical community, physician specialty society recommendations, and expert opinion as relevant to supplement those sources. Brand New Day/Central Health Medicare Plan Medical Policies, MCG™ Guidelines, and the ASAM Criteria™ are not intended to be used without the independent clinical judgment of a qualified health care provider considering the individual circumstances of each member's case. The treating health care providers are solely responsible for diagnosis, treatment, and medical advice. Members may contact Brand New Day/Central Health Medicare Plan Customer Service at the phone number listed on their member identification card to discuss their benefits more specifically. Providers with questions about this Brand New Day/Central Health Medicare Plan Medical Policy may contact the Health Plan. Brand New Day/Central Health Medicare Plan policies and practices are compliant with federal and state requirements, including mental health parity laws.

If there is a difference between this policy and the member specific plan document, the member benefit plan document will govern. For Medicare Advantage members, Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), govern. Refer to the CMS website at http://www.cms.gov for additional information.

Brand New Day/Central Health Medicare Plan medical policies address technology assessment of new and emerging treatments, devices, drugs, etc. They are developed to assist in administering plan benefits and do not constitute an offer of coverage nor medical advice. Brand New Day/Central Health Medicare Plan medical policies contain only a partial, general description of plan or program benefits and do not constitute a contract. Brand New Day/Central Health Medicare Plan does not provide health care services and, therefore, cannot guarantee any results or outcomes. Treating providers are solely responsible for medical advice and treatment of members. Our medical policies are updated based on changes in the evidence and healthcare coding and therefore are subject to change without notice. CPT codes, descriptions and materials are copyrighted by the American Medical Association (AMA). MCG™ and Care Guidelines® are trademarks of MCG Health, LLC (MCG).

PURPOSE

To promote consistency between reviewers in clinical coverage decision-making by providing the criteria that generally determine the medical necessity of Nulibry™ (fosdenopterin hydrobromide) therapy.

POLICY/CRITERIA

Prior Authorization and Medical Review is required.

Coverage for Nulibry will be provided for 12 months and may be renewed (unless otherwise specified).

Molybdenum cofactor deficiency (MoCD) Type A

Initial Therapy

1. Nulibry is prescribed by or in consultation with a pediatrician, geneticist, or a physician who specializes in molybdenum cofactor deficiency (MoCD) Type A; **AND**

2. Patient meets **one** of the following:
    a. Patient has a documented diagnosis of MoCD Type A was confirmed by genetic testing documenting a mutation in the molybdenum cofactor synthesis gene 1 (MOSC1); **OR**
    b. Patient has a presumed diagnosis of MoCD Type A and genetic test results are pending; **AND**
        i. Patient has clinical signs and symptoms associated with MoCD Type A (e.g., encephalopathy, intractable seizures, exaggerated startle response, high-pitched cry, axial hypotonia, limb hypertonia, feeding difficulties, elevated urinary sulfite and/or S-sulfocysteine (SSC), elevated xanthine in urine or blood, or low or absent uric acid in the urine or blood). *****

*****Authorization will be provided for 3 months

Continuation Therapy

1. Patient has received less than 12 months of therapy with Nulibry and has genetic testing results documenting a mutation in the molybdenum cofactor synthesis gene 1 (MOSC1); **OR**
2. Patient has received at least 12 months of therapy and is experiencing benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for encephalopathy, seizure activity, improved or normalized uric acid, urinary S-sulfocysteine, and xanthine levels).

LIMITATIONS/EXCLUSIONS

1. Any indication other than those listed above due to insufficient evidence of therapeutic value

BACKGROUND

Nulibry is indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A.

Patients with MoCD Type A have mutations in the MOCS1 gene leading to deficient MOCS1A/B dependent synthesis of the intermediate substrate, cPMP. Substrate replacement therapy with NULIBRY provides an exogenous source of cPMP, which is converted to molybdopterin. Molybdopterin is then converted to molybdenum cofactor, which is needed for the activation of molybdenum-dependent enzymes, including sulfite oxidase (SOX), an enzyme that reduces levels of neurotoxic sulfites.

DEFINITIONS

1. NULIBRY (fosdenopterin) for injection, for intravenous use. Initial U.S. Approval: 2021
    a. NULIBRY (fosdenopterin) for injection is a white to pale yellow lyophilized powder or cake inn a single-dose clear glass vial for reconstitution.
    b. Each NULIBRY vial contains 9.5 mg of fosdenopterin.

Medical Policy

CODING

Applicable NDC Codes	
73129-0001-01	Nulibry 9.5 mg single-dose vial as a lyophilized powder for injection

Applicable Procedure Code	
J3490	Unclassified drugs (When utilized for Nulibry [fosdenopterin hydrobromide])

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

EVIDENCE BASED REFERENCES

1. Product Information: NULIBRY(TM) intravenous injection, fosdenopterin intravenous injection. Origin Biosciences Inc (per manufacturer), Boston, MA, 2021.

POLICY HISTORY

Original Effective Date	July 19, 2021
Revised Date	• November 1, 2022 – no changes • February 2, 2022: Annual review – no changes made. • February 28, 2023 – Annual Review and approval (no policy revisions made) • March 1, 2023 – Adopted by MA UM Committee (no policy revisions made) • January 1, 2024 - Updated to Brand New Day/Central Health Medicare Plan (no policy revisions made)3
Approval Body	Pharmacy and Therapeutics Committee

Approved by Pharmacy and Therapeutics Committee on 2/28/2023