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Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	March 26, 2021
Subject:	Nulibry	**Page:**	1 of 4

Last Review Date: March 8, 2024

Nulibry

Description

Nulibry (fosdenopterin)

Background

Nulibry (fosdenopterin) is a cyclic pyranopterin monophosphate (cPMP). Patients with molybdenum cofactor deficiency (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, an enzyme that reduces levels of neurotoxic sulfites (1).

Regulatory Status

FDA-approved indication: Nulibry is a cyclic pyranopterin monophosphate (cPMP) indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A (1).

Nulibry has phototoxic potential. Nulibry-treated patients or their caregivers should be advised to avoid or minimize patient exposure to direct sunlight and artificial UV light exposure and to adopt precautionary measures (e.g., protective clothing and hats, broad spectrum sunscreen in patients 6 months of age and older, etc). If photosensitivity occurs, caregivers/patients should be advised to seek medical attention immediately and consider a dermatological evaluation (1).

The safety and effectiveness of Nulibry for the treatment of MoCD Type A have been established in pediatric patients starting from birth (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Nulibry may be considered **medically necessary** if the conditions indicated below are met.

Nulibry may be considered **investigational** for all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

Molybdenum cofactor deficiency (MoCD) Type A

AND ALL of the following:

1. Diagnosis has been confirmed by genetic testing **OR** test sample has been submitted for genetic testing prior to starting treatment
    a. If the diagnosis of MoCD Type A is **NOT** confirmed by future genetic testing, Nulibry will be discontinued
2. Patient and/or caregiver will be advised to avoid or minimize patient exposure to direct sunlight and artificial UV light and to take precautionary measures (e.g., protective clothing and hats, broad-spectrum sunscreen in patients 6 months of age and older, etc)

Prior – Approval *Renewal* Requirements

Diagnosis

Patient must have the following:

Molybdenum cofactor deficiency (MoCD) Type A

AND the following:

1. Patient and/or caregiver will be advised to avoid or minimize patient exposure to direct sunlight and artificial UV light and to take precautionary measures (e.g.,

protective clothing and hats, broad-spectrum sunscreen in patients 6 months of age and older, etc)

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Nulibry (fosdenopterin) is a cyclic pyranopterin monophosphate (cPMP). Patients with molybdenum cofactor deficiency (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, an enzyme that reduces levels of neurotoxic sulfites. The safety and effectiveness of Nulibry for the treatment of MoCD Type A have been established in pediatric patients starting from birth (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Nulibry while maintaining optimal therapeutic outcomes.

References

1. Nulibry [package insert]. Boston, MA: Origin Biosciences, Inc.; October 2022.

Policy History

Date	Action
March 2021	Addition to PA
June 2021	Annual review. Revised genetic testing requirement per SME: “Diagnosis has been confirmed by genetic testing **OR** test sample has been submitted

	for genetic testing prior to starting treatment”
September 2022	Annual review
September 2023	Annual review and reference update
March 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.