

Clinical Policy: Fosdenopterin (Brand Name)

Reference Number: CP.PHAR.471

Effective Date: **FDA Approval Date**

Last Review Date: 05.20

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Fosdenopterin (**Brand Name^{®/TM}**) is a cyclic pyranopterin monophosphate (cPMP) replacement therapy.

FDA Approved Indication(s) [Pending]

Fosdenopterin is indicated for the treatment of pediatric patients with molybdenum cofactor deficiency (MOCD) type A.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Centene Corporation[®] that fosdenopterin is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Molybdenum Cofactor Deficiency Type A (must meet all):

1. Diagnosis of MOCD type A confirmed by genetic testing (i.e., presence of molybdenum cofactor synthesis gene 1 (MOSC1) mutation);*
2. Prescribed by or in consultation with a neurologist or specialist with expertise in the management of MOCD (e.g., pediatric geneticist);
3. Age ≤ 18 years;*
4. Documentation of baseline urinary s-sulfocysteine (SSC), xanthine, and uric acid levels;
5. Dose does not exceed the FDA approved maximum dose.*

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Molybdenum Cofactor Deficiency Type A (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by, including but not limited to, improvement in any of the following parameters:
 - a. Clinical outcomes, such as: improved symptoms, achievement of motor milestones, decreased seizure activity, lack of clinical deterioration (e.g., no progression to severe epileptic encephalopathy);
 - b. Biochemical outcomes, such as: decreased or normalized urinary SSC or xanthine levels, increased or normalized uric acid levels;
3. If request is for a dose increase, new dose does not exceed the FDA approved maximum dose.*

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

- A.** Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

cPMP: cyclic pyranopterin monophosphate

FDA: Food and Drug Administration

MOCD: molybdenum cofactor deficiency

MOSC1: molybdenum cofactor synthesis gene 1

SSC: s-sulfocysteine

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

- Contraindication(s): **pending**
- Boxed warning(s): **pending**

Appendix D: General Information

- A list of available genetic tests for MOCD type A can be found here:
<https://www.ncbi.nlm.nih.gov/gtr/all/tests/?term=C1854988&filter=testtype:clinical>.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
MOCD type A*	IV; pending	Pending

VI. Product Availability [Pending]
Pending

VII. References

1. ClinicalTrials.gov. Study of ORGN001 (formerly ALXN1101) in neonates with molybdenum cofactor deficiency (MOCD) type A. Available at: <https://clinicaltrials.gov/ct2/show/NCT02629393>. Accessed February 7, 2020.
2. ClinicalTrials.gov. Safety & efficacy study of ORGN001 (formerly ALXN1101) in pediatric patients with MoCD type A currently treated with rcPMP. Available at: <https://clinicaltrials.gov/ct2/show/NCT02047461>. Accessed February 7, 2020.
3. Schwahn BC, Van Spronsen FJ, Belaidi AA, et al. Efficacy and safety of cyclic pyranopterin monophosphate substitution in severe molybdenum cofactor deficiency type A: a prospective cohort study. Lancet. 2015; 386: 1955-1963.
4. Spiegel R, Schwahn B, Scribner CL, Confer N. A natural history study of molybdenum cofactor (MoCo) and isolated sulfite oxidase deficiencies (ISOD). Poster presented at the 2019 Society for the Study of Inborn Errors of Metabolism (SSIEM); September 3-6, 2019; Rotterdam, The Netherlands. Available at: https://www.origintx.com/wp-content/uploads/SSIEM-502_MoCD-Poster_27Aug19-002-Final.pdf. Accessed February 7, 2020.
5. U.S. National Library of Medicine, Genetics Home Reference. Molybdenum cofactor deficiency. Reviewed March 2014. Available at: <https://ghr.nlm.nih.gov/condition/molybdenum-cofactor-deficiency>. Accessed February 7, 2020.

Coding Implications [Pending]

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
Pending	Pending

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	03.03.20	05.20

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in

developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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