Dojolvi (triheptanoin)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations, or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Dojolvi is indicated as a source of calories and fatty acids for the treatment of pediatric and adult patients with molecularly confirmed long-chain fatty acid oxidation disorders (LC-FAOD).

POLICY

Required Documentation

Submission of the following information is necessary to initiate the prior authorization review:

A. Chart note documentation of at least one hospitalization or ER visit within the past year due to rhabdomyolysis, cardiomyopathy, or hypoglycemic episodes.
B. Chart or laboratory documentation of low enzyme activity in cultured fibroblasts and/or pathogenic mutations confirmed by genetic testing as required in the Criteria for Initial Approval.

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

Criteria for Initial Approval

Long-chain fatty acid oxidation disorders (LC-FAOD)

Authorization of **6 months** may be granted for treatment of long-chain fatty acid oxidation disorders when all of the following criteria are met:

A. Member has a diagnosis of carnitine palmitoyltransferase type 1 (CPT1) deficiency, carnitine palmitoyltransferase type 2 (CPT2) deficiency, carnitine-acylcarnitine translocase (CACT) deficiency, very-long-chain acyl-CoA dehydrogenase (VLCAD) deficiency, long-chain L-3 hydroxyacyl-CoA dehydrogenase deficiency (LCHAD) or trifunctional protein (TFP) deficiency
B. Member has been receiving a low-fat/high-carbohydrate diet and medium-chain triglyceride (MCT) supplementation (e.g., MCT oil supplements, specialized infant or pediatric formula supplemented with MCT for LC-FAOD such as Lipistart, Monogen, Portagen, Enfaport, MCT Procal, MCT Oil, and Liquigen).
C. Member has experienced at least one hospitalization or ER visit within the past year due to any of the following events: rhabdomyolysis, cardiomyopathy, or hypoglycemic episodes.
D. At least two of the following diagnostic criteria are met:
    1. Elevated acylcarnitine level on a newborn blood spot or in plasma, as applicable to the specific disease:
        a. CPT2 and CACT deficiency: elevated C16 and/or C18:1
        b. CPT1 deficiency: elevated C0: C0/C16 + C18:1
        c. LCHAD and TFP deficiency: elevated C16-OH and/or C18 and other acylcarnitines
        d. VLCAD deficiency: elevated C14:1 and/or other long-chain acylcarnitines
    2. Low enzyme activity in cultured fibroblasts
    3. One or more known pathogenic mutations in CPT1A, SLC25A20, CPT2, acyl-CoA dehydrogenase very-long-chain (ACADVL), hydroxyacyl-CoA dehydrogenase trifunctional multienzyme complex subunit alpha (HADHA) or hydroxyacyl-CoA dehydrogenase trifunctional multienzyme complex subunit beta (HADHB) gene

Continuation of Therapy

Authorization of **12 months** may be granted for members with an indication listed in the Criteria for Initial Approval who are currently receiving the requested medication through a paid pharmacy or medical benefit, and who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in cardiomyopathy, glycemic control or exercise tolerance, or a reduction in episodes of cardiomyopathy, rhabdomyolysis, hypoglycemia or hospitalizations).

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- N/A

REFERENCES

- Dojolvi [package insert]. Novato, CA; Ultragenyx Pharmaceutical Inc.; October 2023.
- Vockley J, Burton B, Berry GT, et al. Results from a 78-week, single-arm, open-label phase 2 study to evaluate UX007 in pediatric and adult patients with severe long-chain fatty acid oxidation disorders (LC-FAOD). J Inherit Metab Dis 2019; 42:169.
- Vockley J, Burton B, Berry GT, et al. UX007 for the treatment of long chain-fatty acid oxidation disorders: Safety and efficacy in children and adults following 24 weeks of treatment. Mol Genet Metab 2017;120:370-377.

- Vockley J, Burton B, Berry G, et al. Effects of triheptanoin (UX007) in patients with long-chain fatty acid oxidation disorders: Results from an open-label, long-term extension study. J Inherit Metab Dis. 2021; 44(1):253-263.
- Gillingham MB, Heitner SB, Martin J, et al. Triheptanoin versus trioctanoin for long-chain fatty acid oxidation disorders: a double blinded, randomized controlled trial. J Inherit Metab Dis. 2017;40(6):831-843.
- Merritt JL 2nd, Norris M, Kanungo S. Fatty acid oxidation disorders. Ann Transl Med. 2018;6(24):473.
- American College of Medical Genetics and Genomics. ACT Sheet and Algorithms. Available at https://www.acmg.net/ACMG/Medical-Genetics-Practice-Resources/ACT_Sheets_and_Algorithms/ACMG/Medical-Genetics-Practice-Resources/ACT_Sheets_and_Algorithms.aspx?hkey=9d6bce5a-182e-42a6-84a5-b2d88240c508. Accessed November 12, 2024.

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POLICY HISTORY

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