Reference number(s)
4024-A

Specialty Guideline Management
Dojolvi

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Dojolvi	triheptanoin

Indications

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

FDA-approved Indications[1]

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

All other indications are considered experimental/investigational and not medically necessary.

Documentation

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

- Chart note documentation of at least one hospitalization or ER visit within the past year due to rhabdomyolysis, cardiomyopathy, or hypoglycemic episodes.
- Chart or laboratory documentation of low enzyme activity in cultured fibroblasts and/or pathogenic variant(s) confirmed by genetic testing as required in the coverage criteria section.

Prescriber Specialties

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

Coverage Criteria

Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)[1-7]

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

- 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.
- 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).
- 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.
- At least two of the following diagnostic criteria are met:
    - Elevated acylcarnitine level on a newborn blood spot or in plasma, as applicable to the specific disease:
        - CPT2 and CACT deficiency: elevated C16 and/or C18:1
        - CPT1 deficiency: elevated C0; C0/C16 + C18
        - LCHAD and TFP deficiency: elevated C16-OH and/or C18:1-OH and/or other long-chain acylcarnitines
        - VLCAD deficiency: elevated C14:1 and/or other long-chain acylcarnitines
    - Low enzyme activity in cultured fibroblasts.
- One or more known pathogenic variant(s) 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 coverage criteria section 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).

References

1. Dojolvi [package insert]. Novato, CA; Ultragenyx Pharmaceutical Inc.; October 2023.
2. 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.
3. 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.
4. 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.
5. 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.
6. Merritt JL 2nd, Norris M, Kanungo S. Fatty acid oxidation disorders. Ann Transl Med. 2018;6(24):473.
7. 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 13, 2024.