CVS Caremark®

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| 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 Indications1

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.