

PHARMACY POLICY STATEMENT

Marketplace

DRUG NAME	Dojolvi (triheptanoin)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Dojolvi, approved by the FDA in 2020, is a medium-chain triglyceride (MCT) 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).

LC-FAODs are inherited metabolic disorders in which the body is unable to convert dietary long-chain fatty acids into energy during times of fasting and physiologic stress. Dojolvi bypasses the long-chain FAOD enzyme deficiencies for energy production and replacement. MCTs do not require long-chain fatty acid oxidation for metabolism and can enter the mitochondria directly. Very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency is the most common LC-FAOD.

Dojolvi (triheptanoin) will be considered for coverage when the following criteria are met:

Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

For **initial** authorization:

- Medication must be prescribed by or in consultation with a metabolic or genetics specialist or dietician; AND
- Chart notes must show the member has a molecularly confirmed diagnosis of an LC-FAOD (e.g., Very long-chain acylCoA dehydrogenase (VLCAD) Deficiency, Carnitine Palmitoyltransferase II (CPT II) Deficiency, Long-chain 3-hydroxyacylCoA dehydrogenase (LCHAD) deficiency, Mitochondrial Trifunctional Protein (TFP) Deficiency), with at least TWO of the following:
 - Disease specific elevation of acylcarnitines on a newborn blood spot or in plasma
 - Low enzyme activity in cultured fibroblasts
 - One or more known pathogenic mutations in ACADVL, CPT2, HADHA, or HADHB; AND
- Member is symptomatic despite dietary management (e.g., low-fat, high carbohydrate diet) and MCT oil (medical food) for at least 90 days, unless contraindicated; AND
- Member does not have pancreatic insufficiency; AND
- Member will discontinue any other medium-chain triglyceride products before starting Dojolvi.
- Dosage allowed/Quantity limit:** See package insert for titration details and equation for dose calculations based on individual's daily caloric intake (DCI). Increase up to a total daily dose of 35% DCI.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

- Chart notes must show improvement per 1 or more of the following parameters:
 - Reduced frequency or severity of major clinical events related to hypoglycemia, cardiomyopathy, and/or rhabdomyolysis
 - Increased endurance and/or exercise tolerance (e.g., 12-minute walk test).

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Dojolvi (triheptanoin) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
09/25/2020	New policy for Dojolvi created.
05/01/2023	Transferred to new template. Added references. Expanded diagnostic criteria. Added dietician as specialist. Changed 6MWT to 12MWT in renewal section.

References:

1. Dojolvi (triheptanoin) [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; 2021.
2. Vockley J, Burton B, Berry G, 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. *Molecular Genetics and Metabolism*. 2017;120(4):370-377. doi:10.1016/j.ymgme.2017.02.005
3. 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(1):169-177. doi:10.1002/jimd.12038
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*. September 2020. doi:10.1002/jimd.12313
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. doi:10.1007/s10545-017-0085-8
6. Knottnerus SJG, Bleeker JC, Wüst RCI, et al. Disorders of mitochondrial long-chain fatty acid oxidation and the carnitine shuttle. *Rev Endocr Metab Disord*. 2018;19(1):93-106. doi:10.1007/s11154-018-9448-1
7. Merritt JL 2nd, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med*. 2018;6(24):473. doi:10.21037/atm.2018.10.57
8. Merritt JL, Macleod E, Jurecka A, Hainline B. Clinical manifestations and management of fatty acid oxidation disorders. *Reviews in Endocrine and Metabolic Disorders*. July 2020. doi:10.1007/s11154-020-09568-3
9. Baker JJ, Burton BK. Diagnosis and Clinical Management of Long-chain Fatty-acid Oxidation Disorders: A Review. *touchREV Endocrinol*. 2021;17(2):108-111. doi:10.17925/EE.2021.17.2.108
10. Vockley J. Long-chain fatty acid oxidation disorders and current management strategies. *Am J Manag Care*. 2020;26(7 Suppl):S147-S154. doi:10.37765/ajmc.2020.88480
11. Zand D, Doan J, Yi S, et al. Regulatory news: Dojolvi (triheptanoin) as a source of calories and fatty acids in long-chain fatty acid oxidation disorders: FDA approval summary. *J Inherit Metab Dis*. 2021;44(3):515-517. doi:10.1002/jimd.12377

Effective date: 10/01/2023

Revised date: 05/01/2023