

## PHARMACY POLICY STATEMENT

### Nevada Medicaid

<b>DRUG NAME</b>	<b>Dojolvi (triheptanoin)</b>
<b>BENEFIT TYPE</b>	Pharmacy
<b>STATUS</b>	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**:

1. Chart notes must show improvement per 1 or more of the following parameters:
  - a) Reduced frequency or severity of major clinical events related to hypoglycemia, cardiomyopathy, and/or rhabdomyolysis
  - b) 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
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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
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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

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