

Effective Date: 06/01/2021
Reviewed: 03/2021, 2/2022, 3/2023, 2/2024
Scope: Medicaid

## SPECIALTY GUIDELINE MANAGEMENT

### DOJOLVI (triheptanoin)

#### POLICY

##### I. 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<sup>1</sup>

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).

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

##### II. 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 Section III.

##### III. CRITERIA FOR INITIAL APPROVAL

##### **Long-chain fatty acid oxidation disorders (LC-FAOD)**<sup>1-5</sup>

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 2 (CPT2) deficiency, very-long-chain acyl-CoA dehydrogenase (VLCAD) deficiency, long-chain L-3 hydroxyacyl-CoA dehydrogenase deficiency (LCHAD) or trifunctional protein (TFP) deficiency.
- B. Medication is being prescribed by or in consultation with a metabolic specialist.
- C. 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).
- D. 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.
- E. 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:
    - i. CPT2 deficiency: elevated C16 and/or C18:1
    - ii. LCHAD and TFP deficiency: elevated C16-OH and/or C18 and other acylcarnitines
    - iii. 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 CPT2, acyl-CoA dehydrogenase very-long-chain (ACADVL), hydroxyacyl-CoA dehydrogenase trifunctional multienzyme complex subunit alpha

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(HADHA) or hydroxyacyl-CoA dehydrogenase trifunctional multienzyme complex subunit beta (HADHB) gene

#### IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for members with documented diagnosis of carnitine palmitoyltransferase type 2 (CPT2) deficiency, very-long-chain acyl-CoA dehydrogenase (VLCAD) deficiency, long-chain L-3 hydroxyacyl-CoA dehydrogenase deficiency (LCHAD) or trifunctional protein (TFP) deficiency who are currently receiving the requested medication through a paid pharmacy 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).

#### V. REFERENCES

1. Dojolvi [package insert]. Novato, CA; Ultragenyx Pharmaceutical Inc.; June 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. Merritt JL 2nd, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med*. 2018;6(24):473.
5. 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.aspx](https://www.acmg.net/ACMG/Medical-Genetics-Practice-Resources/ACT_Sheets_and_Algorithms.aspx). Accessed July 20, 2020.