Effective Date: 06/01/2021
Reviewed: 03/2021, 2/2022, 3/2023, 2/2024, 2/2025 Scope: Medicaid

SPECIALTY GUIDELINE MANAGEMENT

DOJOLVI (triheptanoin)

POLICY

I. INDICATIONS

The indications below include 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 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. CRITERIA FOR INITIAL APPROVAL

Long-chain fatty acid oxidation disorders (LC-FAOD)[1-5]
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. Documentation that the 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. Documentation that the 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. Documentation that the 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 and documentation is provided for:
    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 (HADHA) or hydroxyacyl-CoA dehydrogenase trifunctional multienzyme complex subunit beta (HADHB) gene

III. 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).

IV. 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. Accessed July 20, 2020.