Pharmacy Policy Bulletin: J-0274 Dojolvi (triheptanoin) – Commercial and Healthcare Reform	
Number: J-0274	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1., 2., or 3.):** **1.** Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-0274-007	**Original Date:** 08/05/2020
Effective Date: 10/08/2025	**Review Date:** 09/17/2025

Drugs Product(s):	• Dojolvi (triheptanoin)
FDA-Approved Indication(s):	• Treatment of pediatric and adult patients with molecularly confirmed long-chain fatty acid oxidation disorders (LC-FAOD)

Background:	• LC-FAODs are autosomal recessive disorders affecting one in approximately 9,300 births. In LC-FAODs, there is an inability to utilize dietary fat as an energy source, resulting in energy deprivation and impairment of cardiac and skeletal muscle. Disease symptoms include frequent myalgia, exercise-induced rhabdomyolysis, infection, and decreased quality of life.
	• Dojolvi is a triglyceride composed of three seven-carbon fatty acids; oxidation of those fatty acids allows the citric acid cycle to proceed.
	• Molecular confirmation of LC-FAODs may be obtained through newborn screening using one of the following genetic tests:
	  ○ Carnitine Palmitoyl transferase type 1A (CPT1A) deficiency - mutations in the ***CPT1A*** gene
	  ○ Carnitine-acylcarnitine translocase (CACT) deficiency - mutations in the ***SLC25A20*** gene
	  ○ Carnitine Palmitoyl transferase type 2 (CPT2) deficiency - mutations in the **CPT2** gene
	  ○ Very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency – mutations in the ***ACADVL*** gene
	  ○ Mitochondrial trifunctional protein (MTP) deficiency/trifunctional protein (TFP) deficiency – mutation in one or both genes: ***HADHA*** and ***HADHB***
	  ○ Long-chain 3-Hydroxyacyl-CoA dehydrogenase (LCHAD) deficiency – mutations in the **HADHA** gene
	• Biochemical tests for LC-FAODs may include tandem mass spectrometry, plasma acylcarnitine profile, and free carnitine levels.

	• Current standard of care for LC-FAODs may include dietary management (e.g. low fat, high carbohydrate diet, avoidance of fasting, supplementation with carnitine and/or medium chain triglyceride [MCT] oil).
	• Commercially available, non-FDA approved MCT oils include products such as Lipistart, Monogen, Portagen, Enfaport, MCT Procal, MCT oil, and Liquigen.
	• ICD-10 Code Information:
	  ○ ICD-10 codes: E71.30 "Disorder of fatty-acid metabolism, unspecified", E71.31 "Disorders of fatty-acid oxidation", E71.310 "Long chain/very long chain acyl CoA dehydrogenase deficiency", and E71.318 "Other disorders of fatty-acid oxidation" may apply to Dojolvi; however, the prescriber must confirm that the member has a specific diagnosis of LC-FAOD.
	• Prescribing Considerations:
	  ○ All patients treated with Dojolvi should be under the care of a clinical specialist knowledgeable in appropriate disease-related dietary management based upon current nutritional recommendations.
	  ○ Dose orally daily to a target of 35% of the patient's total prescribed daily caloric intake (DCI) as calculated: $Total\ daily\ dose\ (\_\ mL) = [Patient's\ DCI\ \_\ kcal) * Target\ \_\%\ dose\ of\ DCI] \div [8.3 \frac{kcal}{mL} of\ Dojolvi]$
	  ○ Divide into at least four doses and administer with meals or snacks.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Dojolvi may be approved when all of the following criteria are met **(A. through E.)**:

A. The member has a diagnosis of LC-FAOD (No ICD-10 Code).

B. The prescriber submits documentation (i.e., chart notes) that the member has a molecularly-confirmed gene mutation with one (1) of the following **(1. through 6.)**:

1. Carnitine Palmitoyl transferase type 1A (CPT1A) deficiency

2. Carnitine-acylcarnitine translocase (CACT) deficiency

3. Carnitine Palmitoyl transferase type 2 (CPT2) deficiency

4. Very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency

5. Mitochondrial trifunctional protein (MTP) deficiency/trifunctional protein (TFP) deficiency

6. Long-chain 3-Hydroxyacyl-CoA dehydrogenase (LCHAD) deficiency

C. The prescriber attests that the member is utilizing dietary management.

D. The member meets one (1) of the following **(1. or 2)**:

1. The prescriber attests that the member has experienced therapeutic failure, contraindication, or intolerance to commercially available medium chain triglyceride (MCT) products.

2. The prescriber attests that the member has experienced at least one (1) significant clinical manifestation of LC-FAOD (e.g., rhabdomyolysis, cardiomyopathy, hypoglycemia).

E. The prescriber attests that Dojolvi will not be used concomitantly with another MCT product.

II. Reauthorization

When a benefit, reauthorization of Dojolvi may be approved when all of the following criteria are met **(A. and B.)**:

A. The prescriber attests that the member has experienced positive clinical response to therapy.

B. The prescriber attests that Dojolvi will not be used concomitantly with another MCT product.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.
II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Dojolvi [package insert]. Novato, California: Ultragenyx Pharmaceutical, Inc.; October 2023.
2. Lindner M, Hoffmann GF, Matern D. Newborn Screening for Disorders of Fatty-Acid Oxidation: Experience and Recommendations From an Expert Meeting. *J Inherit Metab Dis*. 2010 Oct;33(5):521-6.
3. Merritt JL, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med*. 2018 Dec; 6(24):473.
4. 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 Nov; 40(6):831–843.
5. Vockley J, Marsden D, McCracken E, et al. Long-Term Major Clinical Outcomes in Patients With Long Chain Fatty Acid Oxidation Disorders Before and After Transition to Triheptanoin Treatment—A Retrospective Chart Review. *Mol Genet Metab*. 2015;116(0):53–60.
6. Merritt JL, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med*. 2018 Dec; 6(24):473.
7. Vockley J, Burton BK, Berry G, et al. Triheptanoin for the treatment of long-chain fatty acid oxidation disorders: Final results of an open-label, long-term extension study. *J Inherit Metab Dis*. 2023 Sept; 46(5): 943-955.
8. Vianey-Saban C, Guffon N, Fouilhoux A, Acquaviva C. Fifty years of research on mitochondrial fatty acid oxidation disorders: The remaining challenges. *J Inherit Metab Dis*. 2023 Sept; 46(5): 848-873.