MEDICAL MUTUAL®

# Drug Policy

| Policy: | Dojolvi (triheptanoin) | Annual Review Date: 08/15/2024<br>Last Revised Date: 08/15/2024 |
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## OVERVIEW

Dojolvi is a medium-chain triglyceride indicated as a source of calories and fatty acids for the treatment of molecularly confirmed long-chain fatty acid oxidation disorders (LC-FAOD) in pediatric and adult patients. Fatty acid oxidation disorders (FAOD) are inborn errors of metabolism resulting in failure of transport of fatty acids into mitochondria. FAOD's are rare life-threatening autosomal disorders characterized by metabolic deficiencies in which the body is unable to convert long-chain fatty acids into energy leading to a variety of clinical presentations. Symptoms typically occur during the neonatal period and infancy.

## POLICY STATEMENT

This policy involves the use of Dojolvi. Prior authorization is recommended for pharmacy benefit coverage of Dojolvi. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Dojolvi as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Dojolvi be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

## RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Dojolvi is recommended in those who meet the following criteria:

1. **Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD), initial therapy**
   **Criteria.** *Patient must meet the following criteria*
   - **A.** The diagnosis of LC-FAOD has been molecularly confirmed by at least TWO of the following diagnostics [documentation required]:
     a. Disease specific elevation of acylcarnitines on a newborn blood spot or in plasma; AND/OR
     b. Low enzyme activity in cultured fibroblasts; AND/OR
     c. Genetic testing showing one or more known pathogenic mutations in CPT2, ACADVL, HADHA, or HADHB; AND

B. The patient has a history of clinical manifestations of LC-FAOD, including one episode of at least ONE of the following, unless diagnosed by newborn screen (NBS) [documentation required]:
    a. Hypoglycemia
    b. Hepatopathy
    c. Skeletal myopathy
    d. Rhabdomyolysis
    e. Cardiomyopathy; AND
C. Dojolvi is prescribed by or in consultation with a specialist in genetic metabolic disorders; AND
D. The patient will not use any other medium-chain triglyceride products concomitantly with Dojolvi;
E. The patient does not have pancreatic insufficiency; AND
F. The dosage of Dojolvi will not exceed 35% of the patient's total daily caloric intake (DCI): AND
G. The patient meets one of the following:
    a. Currently managed on a stable treatment regiment, which may include a low-fat, high-carbohydrate diet, avoidance of fasting, and/or medium-chain triglyceride (MCT) oil; OR
    b. Newly diagnosed with LC-FAOD

**2. <u>Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD), continuation of therapy</u>**
**Criteria.** *Patient must meet the following criteria*

**A.** Dojolvi is prescribed by or in consultation with a specialist in genetic metabolic disorders; AND
**B.** The patient has documented clinical improvement, per the prescribing physician (e.g. gross motor development/motor function for infants and young children, exercise tolerance and endurance for older children and adults, and a decrease in the frequency of major medical episodes of hypoglycemia, rhabdomyolysis, and exacerbation of cardiomyopathy).

**Initial Approval/ Extended Approval.**
**A)** *Initial Approval:* 6 months
**B)** *Extended Approval:* 6 months

## CONDITIONS NOT RECOMMENDED FOR APPROVAL

Dojolvi has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Concomitant Use of Another Medium-Chain Triglyceride (MCT) Product.** Patients receiving another MCT product must discontinue the MCT product prior to use of Dojolvi.

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

**Documentation Requirements:**

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

## REFERENCES

1. Dojolvi™ oral liquid [prescribing information]. Novato, CA: Ultragenyx; November 2021.
2. Merritt JL II, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med.* 2018;6(24):473.
3. Knotterus SJG, Bleeker JC, Wüst RCI, et al. Disorders of mitochondrial long-chain fatty acid oxidation and the carnitine shuffle. *Rev Endocr Metab Disord.* 2018;19:93-106.
4. 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(4):370-77.
5. ACT Sheets and Algorithms: Newborn Screening ACT Sheets and Algorithms. American College of Molecular Genetics and Genomics. Available at: https://www.acmg.net/ACMG/Medical-Genetics-Practice-Resources/ACT_Sheets_and_Algorithms.aspx. Accessed on July 18, 2023.
6. Spiekerkoetter U, Lindner M, Santer R, et al. Treatment recommendations in long-chain fatty acid oxidation defects: consensus from a workshop. *J Inherit Metab Dis.* 2009;32(4):498-505.