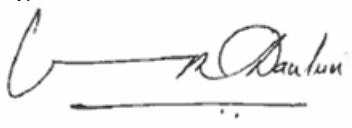


## Prior Authorization Review Panel

### CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.  
Policies submitted without this form will not be considered for review.

<b>Plan: PA Health &amp; Wellness</b>	<b>Submission Date: 11/01/2022</b>
<b>Policy Number: PA.CP.PHAR.509</b>	<b>Effective Date: 10/2020</b> <b>Revision Date: 10/2022</b>
<b>Policy Name: Triheptanoin (Dojolvi)</b>	
<p><b>Type of Submission – <u>Check all that apply</u>:</b></p> <p> <input type="checkbox"/> New Policy  <input checked="" type="checkbox"/> Revised Policy*  <input type="checkbox"/> Annual Review - No Revisions  <input type="checkbox"/> <b>Statewide PDL</b> - <i>Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.</i> </p>	
<p><b>*All revisions to the policy <u>must</u> be highlighted using track changes throughout the document.</b></p> <p><b>Please provide any changes or clarifying information for the policy below:</b></p> <p>4Q 2022 annual review: no significant changes; references reviewed and updated</p>	
<b>Name of Authorized Individual (Please type or print):</b>  <b>Venkateswara R. Davuluri, MD</b>	<b>Signature of Authorized Individual:</b> 

## Clinical Policy: Triheptanoin (Dojolvi)

Reference Number: PA.CP.PHAR.509

Effective Date: 10/2020

Last Review Date: 10/2022

[Coding Implications](#)  
[Revision Log](#)

### Description

Triheptanoin (Dojolvi<sup>TM</sup>) is medium-chain triglyceride.

### FDA Approved Indication(s)

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).

### Policy/Criteria

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

It is the policy of health plans affiliated with PA Health & Wellness<sup>®</sup> that Dojolvi is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

##### A. Long-Chain Fatty Acid Oxidation Disorders (must meet all):

1. Diagnosis of a LC-FAOD (*see Appendix D for example of diagnostic criteria*);
2. Prescribed by or in consultation with an endocrinologist, geneticist, or metabolic disease specialist;
3. Documentation of member's daily caloric intake (DCI);
4. Total daily dose does not exceed 35% of the member's DCI (*see Appendix D*).

**Approval duration: 6 months**

##### B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

#### II. Continued Therapy

##### A. Long-Chain Fatty Acid Oxidation Disorders (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy (*see Appendix E*);
3. If request is for a dose increase, new total daily dose does not exceed 35% of the member's DCI (*see Appendix D*).

**Approval duration: 12 months**

##### B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.

- Approval duration: Duration of request or 6 months (whichever is less); or**
- Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

### III. Diagnoses/Indications for which coverage is NOT authorized:

- Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

### IV. Appendices/General Information

#### Appendix A: Abbreviation/Acronym Key

DCI: daily caloric intake

FDA: Food and Drug Administration

LC-FAOD: long-chain fatty acid  
oxidation disorders

MCT: medium-chain triglycerides

#### Appendix B: Therapeutic Alternatives

*This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.*

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
MCT oil	0.5 g/kg/day in three divided doses, which can be gradually increased to 1.0 to 1.5 g/kg per day	Varies

*Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.*

#### Appendix C: Contraindications/Boxed Warnings

None reported

#### Appendix D: General Information

- Diagnosis of LC-FAOD may be confirmed when at least 2 of the following 3 criteria are met:
  - Disease specific elevations of acylcarnitines on a newborn blood spot or in plasma,
  - Low enzyme activity in cultured fibroblasts,
  - One or more known pathogenic gene mutations
- LC-FAOD examples and associated genetic mutation locations:

LC-FAOD examples	Associated Genes
trifunctional protein deficiency (TFPD)	<i>HADHA, HADHB</i>
long-chain 3-hydroxyacyl CoA dehydrogenase deficiency (LCHADD)	<i>HADHA</i>
very long-chain acyl CoA dehydrogenase deficiency (VLCADD)	<i>ACADVL</i>
carnitine palmitoyltransferase-1 or 2 deficiency (CPT1/2D)	<i>CPT1A, CPT2</i>
carnitine-acylcarnitine translocase deficiency (CACTD)	<i>SLC25A20</i>

- Dosage calculation:
  - Caloric value of Dojolvi = 8.3 kcal/mL

- Round the total daily dosage to the nearest whole number
- Total Daily Dose ( \_\_\_ mL) =  $\frac{\text{Patient's DCI ( \# kcal) } \times \text{Target \# \% dose of DCI}}{8.3 \text{ kcal/mL of Dojolvi}}$

*Appendix E: Response to Therapy*

- In adults, examples may include but are not limited to a reduced incidence of muscle myalgias, rhabdomyolysis, exercise intolerance, cardiac symptoms, hypoglycemia, hepatomegaly symptoms, or vomiting and dehydration precipitated by infections.
- In pediatrics, early treatment may not prevent symptomatology. Positive response can be demonstrated by continued tolerance to Dojolvi administration.

**V. Dosage and Administration**

Indication	Dosing Regimen	Maximum Dose
LC-FAOD	Target daily dosage of Dojolvi is up to 35% of the patient's total prescribed DCI divided into at least four doses and administered at mealtimes or with snacks	35% of patient's DCI

**VI. Product Availability**

Oral liquid: 500 mL (100% w/w of triheptanoin)

**VII. References**

1. Dojolvi Prescribing Information. Novato, CA: Ultragenyx Pharmaceutical Inc. November 2021. Available at: <https://www.ultragenyx.com/wp-content/uploads/2021/11/DOJOLVI-USPI.pdf#page=1>. Accessed July 29, 2022.
2. 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.
3. Yamada K and Taketani T. Management and diagnosis of mitochondrial fatty acid oxidation disorders: focus on very-long-chain acyl-CoA dehydrogenase deficiency. Journal of Human Genetics 2019; 64:73-85.
4. Merrit JL 3<sup>rd</sup>, Norris M, and Kanungo S. Fatty Acid Oxidation Disorders. Ann Transl Med 2018; 6(24):473.

Reviews, Revisions, and Approvals	Date
Policy created	10/2020
4Q 2021 annual review: no significant changes; references reviewed and updated.	10/2021
4Q 2022 annual review: no significant changes; references reviewed and updated.	10/2022