Pharmacy Policy Bulletin

Title: Triheptanoin (Dojolvi™)

Policy #: Rx.01.235

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

Intent:

The intent of this policy is to communicate the medical necessity criteria for **Dojolvi™ (triheptanoin)** as provided under the member's prescription drug benefit.

Description:

Fatty acid oxidation disorders (FAOD) are inborn errors of metabolism due to disruption of either the fatty acid transport using the carnitine transport pathway or mitochondrial beta oxidation. FAODs present differently depending upon the specific disorder, but all ultimately require similar treatment.

FAOD presenting in neonates generally show severe symptoms, including cardiomyopathy. During infancy and childhood, patients experience liver dysfunction and hypoketotic hypoglycemia. After adolescence, episodic rhabdomyolysis is sometimes seen.

General treatment of FAODs include avoidance of fasting, aggressive treatment during illness, and supplementation of carnitine, if necessary. Long chain fatty acid oxidation disorders differ by requiring a fat-restricted diet and supplementation of medium chain triglyceride oil and often docosahexaenoic acid.

Dojolvi™ (triheptanoin) is a medium-chain triglyceride 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:

INITIAL CRITERIA: Triheptanoin (Dojolvi™) is approved when BOTH of the following criteria are met:

1. Diagnosis of molecularly confirmed long-chain fatty acid oxidation disorders (LC-FAOD); and
2. Will be used as a source of calories and fatty acids

Initial Authorization: 2 years

REAUTHORIZATION CRITERIA: Triheptanoin (Dojolvi™) is re-approved when there is documentation of positive clinical response to therapy.

Reauthorization: 2 years

Black Box Warning as shown in the drug Prescribing Information:

N/A

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Dojolvi™ (triheptanoin) [package insert]. Novato, Ca. Ultragenyx Pharmaceutical Inc. September 2020. Available from: accessdata.fda.gov/drugsatfda_docs/label/2020/213687s00lbl.pdf. Accessed March 31,2021.

Merritt JL 2nd, Norris M, Kanungo S. Fatty acid oxidation disorders. *Ann Transl Med.* 2018;6(24):473. Doi:10.21037/atm.2018.10.57. Accessed March 31,2021.

Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Dojolvi™	Triheptanoin

Cross References:

Rx.01.33 Off Label Use

Policy Version Number:	2.0
P&T Approval Date:	March 18, 2021
Policy Effective Date:	July 01, 2021
Next Required Review Date:	March 18, 2022