Dojolvi Prior Authorization Program Summary

This program applies to FlexRx Closed, FlexRx Open, FocusRx, GenRx Closed, GenRx Open, Health Insurance Marketplace, and KeyRx formularies.

This is a FlexRx Standard and GenRx Standard program.

POLICY REVIEW CYCLE

Effective Date	Date of Origin
1/1/2024	1/1/2021

FDA APPROVED INDICATIONS AND DOSAGE

Agent(s)	FDA Indication(s)	Notes	Ref#
Dojolvi® (triheptanoin) Oral liquid	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)		1

See package insert for FDA prescribing information: https://dailymed.nlm.nih.gov/dailymed/index.cfm

CLINICAL RATIONALE

Long Chain Fatty Acid Oxidation Disorders	Long-chain fatty acid oxidation disorders (LCFAOD) is one of the most severe categories of fatty acid oxidation disorders (FAOD) and often present within a few days of life, though milder disease can have an onset in adolescents or adulthood. LCFAOD consists of a family of rare genetic disorders caused by impaired fatty acid metabolism pathways. LCFAOD can manifest with severe symptoms including cardiomyopathy, arrhythmia, skeletal myopathy, rhabdomyolysis, transaminitis, liver failure, and retinal degeneration.(2) FAOD are often captured as part of newborn screenings (NBS). A plasma acylcarnitine profile is necessary for diagnosis following an abnormal NBS. DNA testing is considered standard for confirmation and can be helpful in genotype/phenotype correlations. DNA sequencing may reveal variants of uncertain significance, so further investigation of enzyme activity through fibroblast or lymphocyte testing may provide additional information of functional significance.(2,4,5) Management of FAOD involve prevention of metabolic decompensation which includes avoidance of prolonged fasting and maintenance of a constant energy supply via carbohydrates during catabolism.(2) The diet of individuals with LCFAOD should be high in carbohydrates and low in long-chain fats. Medium chain fatty acid supplements are provided as a source for beta-oxidation.(3)
Safety(1)	Triheptanoin carries no contraindications nor black box warnings. Dojolvi is not compatible with certain plastics and should not be prepared or administered using containers, dosing syringes or measuring cups made of polystyrene or polyvinyl chloride (PVC) plastics.

REFERENCES

Number	Reference
1	Dojolvi prescribing information. Ultragenyx Pharmaceutical Inc. November 2021.
2	Vockley J, et al. Overview of Fatty Acid Oxidation Disorders. UpToDate. Literature review current through June 2023. Last updated April 2022.
3	Vockley J, et al. Specific Fatty Acid Oxidation Disorders. UpToDate. Literature review current through June 2023. Last updated January 2022.
4	Knottnerus SJG, Bleeker JC, Wust RCI, et al. Disorders of Mitochondrial Long-Chain Fatty Acid Oxidation and the Carnitine Shuttle. Rev Endocr Metab Disord. 2018;19(1):93-106.
5	Merritt JL, Norris M, Kanungo S. Fatty Acid Oxidation Disorders. Ann Transl Med. 2018;6(24):473.

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Dojolvi	triheptanoin oral liquid	100 %	M ; N ; O ; Y	N		

CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Dojolvi	triheptanoin oral liquid	100 %	FlexRx Closed ; FlexRx Open ; FocusRx ; GenRx Closed ; GenRx Open ; Health Insurance Marketplace/BasicRx ; KeyRx

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
	Initial Evaluation **Target Agent** will be approved when ALL of the following are met: 1. The patient has ONE of the following: A. The patient has a diagnosis of long-chain fatty acid oxidation disorder (LCFAOD) AND ALL of the following: 1. The diagnosis has been confirmed by at least TWO of the following: A. Disease-specific elevations of acylcarnitines on a newborn blood spot or in plasma B. Enzyme activity assay (in cultured fibroblasts or lymphocytes) demonstrating deficiency of an enzyme associated with LCFAODs C. Genetic testing demonstrating pathogenic mutation in a gene associated with LCFAODs **AND** 2. The patient had symptomatic LCFAOD prior to therapy with the requested agent **AND** 3. The patient will not be concurrently using another medium chain triglyceride product **AND** 4. The patient will not be using the requested agent for more than 35% of the patient's total prescribed daily caloric intake **AND** 5. The requested agent will not be administered using containers or utensils made of polyvinyl chloride (PVC) **OR**

Module	Clinical Criteria for Approval
	B. The patient has another FDA approved indication for the requested agent and route of administration **OR** C. The patient has another indication that is supported in compendia for the requested agent and route of administration **AND** 2. The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist) or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND** 3. The patient does NOT have any FDA labeled contraindications to the requested agent **Length of Approval:** 12 months **Compendia Allowed:** AHFS, or DrugDex 1 or 2a level of evidence **Renewal Evaluation** **Target Agent** will be approved when ALL of the following are met: 1. The patient has been previously approved for the requested agent through the plan's Prior Authorization process **AND** 2. The patient has had clinical benefit with the requested agent **AND** 3. If the patient has a diagnosis of LCFAOD, ALL of the following: A. The patient will not be concurrently using another medium chain triglyceride product **AND** B. The patient will not be using the requested agent for more than 35% of the patient's total prescribed daily caloric intake **AND** C. The requested agent will not be administered using containers or utensils made of polyvinyl chloride (PVC) **AND** 4. The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist) or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND** 5. The patient does NOT have any FDA labeled contraindications to the requested agent **Length of Approval:** 12 months

