DOJOLVI (TRIHEPTANOIN) PREAUTHORIZATION REQUIRED

X.159

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POLICY

Initial Review

I. Dojovli (triheptanoin) may be considered **medically necessary** when the following criteria are met:

A. The patient has one of the following;

1. The patient has a diagnosis of long-chain fatty acid oxidation disorder (LCFAOD) **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 triglycerides 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

B. The patient has another FDA approved indication for the requested agent and route of administration

OR

AND

D. 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

E. The patient does not have any FDA labeled contraindications to the requested agent

Length of approval: 12 months

Renewal Review

I. Dojovli (triheptanoin) may be considered **medically necessary** when the following criteria are met:

A. The patient has been previously approved for the requested agent through BCBSNE's Prior Authorization process **AND**

B. The patient has had clinical benefit with the requested agent **AND**

C. If the patient has a diagnosis of LCFAOD, **ALL** of the following:

1. the patient is practicing prevention of metabolic decompensation **AND**

2. The patient will not be concurrently using another medium chain triglyceride product **AND**

3. The patient will not be using the requested agent for more than 35% of the patient's total prescribed daily caloric intake **AND**

4. The requested agent will not be administered using containers or utensils made of polyvinyl chloride (PVC)

AND

D. The prescriber is a specialist in the area of the patient's diagnosis (e.g. endocrinologist) or the prescriber has consulted with a

E. The patient does not have any FDA labeled contraindications to the requested agent.

Dates

Original Effective
08-12-2020
Last Review
11-06-2024
Next Review
11-11-2025

CLINICAL RATIONALE

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.[3]

FAOD are often captured as part of new born screenings. Additional laboratory and/or genetic testing may be necessary to identify specific variations in FAOD and predict phenotype.[3]

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.[3] 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.[4]

The efficacy of triheptanoin as a source of calories and fatty acids was evaluated in Study 3, a 4-month double-blind randomized controlled study comparing triheptanoin (7-carbon chain fatty acid) with trioctanoin (8-carbon chain fatty acid). The study enrolled 32 adult and pediatric patients with a confirmed diagnosis of LC-FAOD

low enzyme activity in cultured fibroblasts, or one or more known pathogenic mutations in CPT2, ACADVL, HADHA, or HADHB.[1]

Baseline cardiovascular function in both groups was normal and within test/retest variability normally observed in repeated echocardiograms. After 4 months, patients in both groups had similar mean changes from baseline in left ventricular ejection fraction and wall mass on resting echocardiogram and similar maximal heart rates on treadmill ergometry.[1]

Patients in the C7 group increased left ventricular (LV) ejection fraction by 7.4% (p=0.046) while experiencing a 20% (p = 0.041) decrease in LV wall mass on their resting echocardiogram. They also required a lower heart rate for the same amount of work during a moderate-intensity exercise stress test when compared to patients taking C8.[2]

Five patients experienced 7 events of rhabdomyolysis in the triheptanoin group and 4 patients experienced 7 events of rhabdomyolysis in the trioctanoin group.[1]

No differences were observed between triheptanoin and trioctanoin groups in blood markers of metabolism including glucose, insulin, lactate, total serum, ketones, acylcarnitines, and serum-free fatty acid concentrations.[1]

C7 improved LV ejection fraction and reduced LV mass at rest, as well as lowering heart rate during exercise among patients with LC-FAODs.[2]

Safety

Contraindications to tripheptanoin liquid include[1]:

- None

Triheptanoin liquid contains the following Black Box Warnings[1]:

- None

REFERENCES

2017
Gillingham MB, Heitner SB, Martin J, Rose S, Goldstein A, Hassan El-Gharbawy A, Deward S, Lasarev MR, Pollaro J, DeLany JP, Burchill LJ, Goodpaster B, Shoemaker J, Matern D, Harding CO, Vockley J. Triheptanoin versus trioctanoin for long-chain fatty acid oxidation disorders:a double blinded, randomized controlled trial. Journal of Inherited Metabolic Disease. 2017 Sept 04; 40; 831-843. Accessed 8/12/2020.

2020
Overview of fatty acid oxidation disorders. UptoDate. Current through 7/2020. Last updated 1/2020.

2020
Specific fatty acid oxidation disorders. UptoDate. Current through 7/2020. Last updated 1/2020.

REVISIONS

12-05-2023
Policy reviewed at Medical Policy Committee meeting on 11/8/2023 – no changes to policy


BlueCross BlueShield
Nebraska