Dojolvi

Background
Dojolvi (triheptanoin) is a medium-chain triglyceride consisting of three odd-chain 7-carbon length fatty acids (heptanoate) that provide a source of calorie and fatty acids to bypass the long-chain fatty acid oxidation disorder enzyme deficiencies for energy production and replacement (1).

Regulatory Status
FDA-approved indication: Dojolvi 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) (1).

Patient metabolic requirements should be determined by their daily caloric intake (DCI) prior to calculating the dose of Dojolvi. For patients receiving another medium-chain triglyceride (MCT) product, the product should be discontinued prior to the first dose of Dojolvi (1).

Dojolvi should be administered mixed with semi-solid food or liquids orally or enterally via a silicone or polyurethane feeding tube. Dojolvi should not be administered alone to avoid gastrointestinal upset (1).

Pancreatic enzymes hydrolyze triheptanoin and release heptanoate as medium-chain fatty acids in the small intestine. Low or absent pancreatic enzymes may result in reduced absorption of
heptanoate subsequently leading to insufficient supplementation of medium-chain fatty acids. Administration of Dojolvi in patients with pancreatic insufficiency should be avoided (1).

The most common adverse reactions to Dojolvi reported in the pooled safety population of Study 1 and Study 2 were gastrointestinal (GI)-related, and included abdominal pain (abdominal discomfort, abdominal pain, abdominal distention, abdominal pain upper, GI pain; 60%), diarrhea (44%), vomiting (44%), and nausea (14%). If a patient experiences gastrointestinal (GI) adverse reactions, dose reduction should be considered until the symptoms resolve (1).

The safety and effectiveness of Dojolvi have been established in pediatric patients aged birth and older (1).

**Related policies**

**Policy**

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Dojolvi may be considered **medically necessary** in patients with long-chain fatty acid oxidation disorder (LC-FAOD) and if the conditions indicated below are met.

Dojolvi is considered **investigational** for all other indications.

**Prior-Approval Requirements**

**Diagnosis**

Patient must have the following:

- Long-chain fatty acid oxidation disorder (LC-FAOD)

**AND ALL** of the following:

1. Diagnosis of LC-FAOD has been molecularly confirmed
2. Patient will not be using Dojolvi with another medium-chain triglyceride (MCT) product
3. Prescriber agrees to monitor gastrointestinal (GI) adverse reactions and adjust the dose as needed
4. Patients using a feeding tube only: patient or caregiver will be advised to regularly inspect the feeding tube for proper functioning and integrity
5. **NO** pancreatic insufficiency

**Prior – Approval Renewal Requirements**

**Diagnosis**

Patient must have the following:

- Long-chain fatty acid oxidation disorder (LC-FAOD)

**AND ALL** of the following:

1. Patient has had an improvement in symptoms (e.g. less episodes of rhabdomyolysis)
2. Patient will not be using Dojolvi with another medium-chain triglyceride (MCT) product
3. Patients using a feeding tube only: patient or caregiver has been advised to regularly inspect the feeding tube for proper functioning and integrity
4. **NO** pancreatic insufficiency
5. **NO** unacceptable gastrointestinal (GI) toxicity

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration** 12 months

**Prior – Approval Renewal Limits**

Same as above

**Rationale**

**Summary**
Dojolvi (triheptanoin) is a medium-chain triglyceride consisting of three odd-chain 7-carbon length fatty acids (heptanoate) that provide a source of calorie and fatty acids to bypass the long-chain fatty acid oxidation disorder enzyme deficiencies for energy production and replacement. The safety and effectiveness of Dojolvi have been established in pediatric patients aged birth and older (1).

Prior approval is required to ensure the safe, clinically appropriate and cost-effective use of Dojolvi while maintaining optimal therapeutic outcomes.

References

Policy History

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>August 2020</td>
<td>Addition to PA</td>
</tr>
<tr>
<td>September 2020</td>
<td>Annual review</td>
</tr>
<tr>
<td>December 2020</td>
<td>Annual review and reference update. Addition of requirements per FEP: “prescriber agrees to monitor GI adverse reactions and adjust dose as needed” added to initiation and “no unacceptable GI toxicity” added to renewal</td>
</tr>
</tbody>
</table>

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 4, 2020 and is effective on January 1, 2021.