

Pr **DOJOLVI**[®]
Triheptanoin Oral Liquid

ONTARIO

Ultragenyx Canada would like to announce that PrDOJOLVI[®] (triheptanoin) is eligible for public reimbursement in Ontario for patients with LC-FAOD based on the reimbursement criteria detailed below under the Ontario Drug Benefit.

DOJOLVI[®] (triheptanoin) is indicated as a source of calories and fatty acids for the treatment of adult and pediatric patients with long-chain fatty acid oxidation disorders (LC-FAOD).

Please consult the Product Monograph at https://dojolvihcp.ca/wp-content/uploads/pdfs/Dojolvi_Final_PM.pdf for important information on contraindications, warnings and precautions, the conditions of clinical use, adverse reactions, drug interactions, and dosing instructions.

The Product Monograph is also available by calling us at 1-833-388-5872.

The DOJOLVI[®] Reimbursement Criteria for the [Ontario Drug Benefit](#) Are as Follows:

Triheptanoin (DOJOLVI) Initiation Criteria

As a source of calories and fatty acids for the treatment of patients with long-chain fatty acid oxidation disorders (LC-FAOD) who meet the following criteria:

1. Patient presents with one or more acute life-threatening events consistent with LC-FAOD AND
2. Patient meets one of the following circumstances:
 - i. Triheptanoin is being used as second or subsequent line therapy in a patient who has a confirmed diagnosis of one of the types of LC-FAOD (Note 1) who is being treated with a less costly therapy with conventional even-chain MCT oil (e.g., trioctanoin) (Note 3) but is experiencing an inadequate response and requires alternative therapy to conventional even-chain medium-chain triglyceride (MCT) supplementation
 - OR
 - ii. Triheptanoin is being initiated as first line therapy in a patient who has a confirmed diagnosis of one of the types of LC-FAOD (Note 1) who is presenting with acute life-threatening events of LC-FAOD
 - OR
 - iii. Triheptanoin is being used as first line therapy in a patient who presents with acute life-threatening events consistent with a LC-FAOD but who does not have a confirmed diagnosis of LC-FAOD (Note 2)
- AND
3. Patient is under the care of a clinician experienced in the management of LC- FAOD

Notes

1. Provide documentation of genetic, biochemical, molecular, and clinical findings and investigations used to support the type of LC-FAOD diagnosis and the severity of the symptoms impacting the affected organ systems.

Request applications should include newborn testing results, including genetic testing results as available and applicable (e.g., mutations in CPT1A, SLC25A20, CPT2, ACADVL, HADHA, HADHB genes), and may include the following to support the initiation criteria:

- plasma total and free carnitine
- plasma acylcarnitine profile
- urine organic acids
- urine acylglycines
- transaminase levels
- creatine kinase
- glucose and ketone patterns
- ammonia levels
- organ systems impacted and severity, frequency, and duration of events
- treatments used for LC-FAOD, including the name, doses, and duration of use of conventional even-chain MCT oils and carnitine (as applicable) and information related to dietary measures to manage the condition

Requests with a confirmed diagnosis should specify the type of LC-FAOD diagnosis, and renewal requests initiated without a confirmed diagnosis should specify the type of LC-FAOD when the diagnosis is confirmed at the time of renewal:

- i. Carnitine palmitoyltransferase 1 A deficiency (CPT1A)
 - ii. Carnitine palmitoyltransferase 2 deficiency (CPT 2)
 - iii. Carnitine-acylcarnitine translocase (CACT) deficiency
 - iv. Very long-chain acyl-coenzyme A dehydrogenase (VLCAD) deficiency
 - v. Long-chain 3-hydroxyacyl-coenzyme A dehydrogenase (LCHAD) deficiency
 - vi. Trifunctional protein (TFP) deficiency
2. Requests should specify the acute life-threatening events that the patient presents with that are consistent with LC-FAOD and include clinical and biochemical findings of impacted organ systems which support warranted triheptanoin initiation. Consult notes may be provided.
 3. Please refer to Ontario's Inherited Metabolic Diseases (IMD) program for funding of conventional MCT oil in Ontario. (https://www.health.gov.on.ca/en/pro/programs/drugs/funded_drug/fund_inherited_drug.aspx)

Requests for patients who were using therapy with conventional even-chain MCT oil (e.g., trioctanoin) should include information related to adherence and optimization on conventional even-chain MCT oil at the time of symptom presentation.

Exclusion Criteria

1. Triheptanoin is not funded in combination with conventional even-chain medium-chain triglyceride (MCT) oil.
2. Triheptanoin will not be funded as initial first line or subsequent line therapy after conventional even-chain MCT oil in asymptomatic patients with a confirmed diagnosis of LC-FAOD.

Approval duration for initial requests for patients with a confirmed diagnosis of LC-FAOD: 1 year

Approval duration for initial requests for patients without a confirmed diagnosis of LC-FAOD: 7 months

Renewal Criteria

Renewals will be considered for patients meeting ALL of the following criteria:

1. Patient continues to be under the care of a clinician experienced in the management of LC-FAOD.
2. Patient who was initiated on triheptanoin without a confirmed diagnosis of LC- FAOD has subsequently received a confirmed diagnosis established by a specialist in metabolic diseases experienced in the treatment and management of LC-FAOD with the type of LC-FAOD specified, and the genetic and other findings are provided to confirm the diagnosis.
3. Patient is optimized on, and adherent to, appropriate dietary management. (Note: Please provide reasons and management plan if this criterion is not met.)
4. Patient continues to benefit from triheptanoin therapy. Note that patients who were started on triheptanoin as first line for acute life-threatening events based on meeting the above criteria and who did not have a prior use of a less costly even-chained MCT oil are to be transitioned to conventional even-chain MCT oil within a year of initiating treatment with Dojolvi. If this is not considered to be appropriate, prescribers should provide rationale as to why this is not warranted for case-by-case review.

Approval duration for renewals: 1 year

Recommended dosage: Daily dosage of up to 35% of the patient's total prescribed daily caloric intake (DCI) divided into at least four doses. Treatment should be individualized based on disease presentation and other clinical findings.

For more information on applying for DOJOLVI® coverage, we can arrange an appointment at your convenience, or you can visit the [UltraCare website](#). You can also find the [UltraCare Enrolment Form](#) on the UltraCare website.