DOJOLVI®
TRIHEPTANOIN
Oral Liquid

TREATING DIVERSE LC-FAOD PATIENT TYPES

Anna
Initiating DOJOLVI for a newborn with VLCAD deficiency

Chloe
Monitoring progress on DOJOLVI for an adolescent with LCHAD deficiency

Michael
Switching an adult patient with CPT II deficiency to DOJOLVI

LC-FAOD

CPT I | CACT | CPT II | VLCAD | TFP | LCHAD

ENZYME DEFICIENCIES

INDICATION

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

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Feeding Tube Dysfunction

- Feeding tube performance and functionality can degrade over time depending on usage and environmental conditions. In clinical trials, feeding tube dysfunction was reported in patients receiving triheptanoin. The contribution of DOJOLVI cannot be ruled out. Do not administer DOJOLVI in feeding tubes manufactured of polyvinyl chloride (PVC). Regularly monitor the feeding tube to ensure proper functioning and integrity.

Adapted from real patient case studies to represent actual clinical scenarios. Guidance is for illustrative purposes only and should not be taken as medical advice.

CACT=carnitine-acylcarnitine translocase; CPT=carnitine palmitoyltransferase; LC-FAOD=long-chain fatty acid oxidation disorders; LCHAD=long-chain 3-hydroxyacyl-CoA dehydrogenase; TFP=trifunctional protein; VLCAD=very long-chain acyl-CoA dehydrogenase.

Please see additional safety information provided throughout the brochure.

CONSIDER THESE PATIENT CASE STUDIES*

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Initiating
a newborn on DOJOLVI

Anna

- **Age:** 3 weeks
- **Type:** VLCAD deficiency

Patient diagnosis considerations:

- Immediately after birth, Anna presented with signs of poor feeding and lethargy, and she also developed hypoglycemia

Treatment:

- Shortly after birth, the NICU initiated DOJOLVI when an abnormal newborn screening was followed up by acylcarnitine testing and a genetic test, confirming LC-FAOD[1]

Key considerations for infants and newborns:

- DOJOLVI can be mixed thoroughly with soft food, including baby food, and appropriate liquids for oral administration[1]
- The safety and effectiveness of DOJOLVI have been established in pediatric patients[1]
- The neonatal population may require higher fat intake and therefore an increased amount of DOJOLVI. Consider current nutritional recommendations when dosing the neonatal population[1]

After initiating treatment with DOJOLVI in the hospital shortly after birth, how would you approach optimizing the continuum of care with DOJOLVI post discharge?

*Adapted from real patient case studies to represent actual clinical scenarios. Guidance is for illustrative purposes only and should not be taken as medical advice.

RECOMMENDED DOJOLVI DOSAGE: The recommended target daily dosage of DOJOLVI is up to 35% of the patient's total prescribed DCI divided into at least four doses and administered by mixing thoroughly into semi-solid food/liquid or medical food/formula at mealtimes or with snacks. In order to reach a target daily dosage, patients may require an increase in their total fat intake.[1] Please refer to the DOJOLVI Prescribing Information for more information on dosing.

IMPORTANT SAFETY INFORMATION (continued)

WARNINGS AND PRECAUTIONS

Intestinal Malabsorption in Patients with Pancreatic Insufficiency

- 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. Avoid administration of DOJOLVI in patients with pancreatic insufficiency.

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Individualizing DOJOLVI treatment for patients undergoing life transitions

Chloe

- **Age:** 18 years
- **Type:** LCHAD deficiency

Patient presentation:

- Chloe is facing challenges while transitioning to college life. She is skipping meals, changing her sleep habits due to her new academic schedule, and balancing peer pressures with the potential urgency of her disease
- She presents with increased fatigue that has been limiting her participation in gymnastics practice, despite eating more during the past 6 to 9 months
- She is currently maintained on DOJOLVI at 28% of her DCI, which was 2000 kcal the last time her dosage was calculated
- **DOJOLVI dosing can be flexible**,[1] so Chloe's daily dosage of 67.5 mL of DOJOLVI is divided into 5 doses: 4 doses of 13 mL each, taken with meals or snacks throughout the day, plus a fifth dose of 15.5 mL taken before gymnastics practice

Treatment:

- Chloe's DOJOLVI dosing regimen may need to be reevaluated and adjusted to take into consideration her current metabolic and physiological demands[1]
- Remind Chloe about the importance of thoroughly mixing her DOJOLVI doses with appropriate foods or liquids, especially at the start of the day[1]

Key considerations:

- The demands of college life and athletics have changed her daily caloric needs
- In addition, Chloe's height and weight have increased since her last visit. Her dosage has not changed since it was first prescribed for her at age 12

Given Chloe's changing metabolic demands and social pressures, what steps would you take to ensure her regimen is optimized and promote adherence?

DCI=daily caloric intake.

IMPORTANT SAFETY INFORMATION (continued)

ADVERSE REACTIONS

Gastrointestinal (GI)

- The most common GI-related adverse reactions reported in the pooled safety population of Studies 1 and 2 were abdominal pain (abdominal discomfort, abdominal distension, abdominal pain, abdominal pain upper, GI pain) [60%], diarrhea [44%], vomiting [44%], and nausea [14%].

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Switching an adult patient to DOJOLVI

Michael

- **Age:** 34 years
- **Type:** CPT II deficiency

Patient presentation:

- Michael was diagnosed with CPT II deficiency, with confirmation by genetic testing, by a neuromuscular specialist during an emergency room visit 15 years ago
- The specialist recommended Michael take an even-chain medium-chain triglyceride (MCT; 20% DCI of 2200 kcal/day), which he has maintained ever since
- He reports taking even-chain MCT twice daily with meals, occasionally 3 times daily if he remembers

Treatment:

- Independently, Michael has learned about the only FDA-approved therapy for LC-FAOD, which may be used as early as first-line[1]
- His neuromuscular specialist makes the decision to switch Michael from MCT to DOJOLVI[1]

Key considerations:

- Michael is interested in exploring treatment options that have been FDA approved for LC-FAOD[1]

How would you approach a discussion with Michael about switching from MCT to DOJOLVI? And how would you manage Michael's treatment regimen to ensure it is optimized?

IMPORTANT SAFETY INFORMATION (continued)

DRUG INTERACTIONS

Pancreatic Lipase Inhibitors

- Co-administration of triheptanoin with a pancreatic lipase inhibitor (e.g., orlistat) may reduce exposure to the triheptanoin metabolite, heptanoate, and reduce the clinical effect of triheptanoin. Avoid co-administration of DOJOLVI with pancreatic lipase inhibitors.

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DOJOLVI is approved for patients of all ages and all types of LC-FAOD[1]

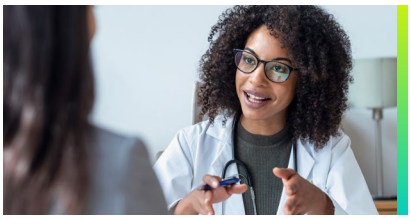
LC-FAOD

CPT I | CACT | CPT II | VLCAD | TFP | LCHAD

ENZYME DEFICIENCIES

- Patients with LC-FAOD require **continuous support and management from their healthcare team**[1]
- **The needs of your patients with LC-FAOD may change** seasonally, over time, and during different stages of life[2-4]

To learn more about DOJOLVI, scan this code



How frequently are you monitoring your patients living with LC-FAOD to address their changing needs?

IMPORTANT SAFETY INFORMATION (continued)

USE IN SPECIFIC POPULATIONS

Pregnancy and Lactation

- There are no available data on triheptanoin use in pregnant women to evaluate for a drug-associated risk of major birth defects, miscarriage, or adverse maternal or fetal outcomes. There is a pregnancy safety study for DOJOLVI. If a patient becomes pregnant while receiving DOJOLVI, healthcare providers should report DOJOLVI exposure by calling Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.
- There are no data on the presence of triheptanoin or its metabolites in human or animal milk, the effects on the breastfed infant, or the effects on milk production. The developmental and health benefits of breastfeeding should be considered along with the clinical need for DOJOLVI and any potential adverse effect on the breastfed infant from DOJOLVI or from the underlying maternal condition.

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DOJOLVI is the first and only FDA-approved treatment for LC-FAOD[1]

- DOJOLVI is indicated for the treatment of **adult and pediatric patients** with LC-FAOD[1]
- As of November 2025, **more than 700 patients have been prescribed DOJOLVI** since its approval in June 2020[5]

Consider DOJOLVI for all your patients diagnosed with LC-FAOD.[1] To learn more, scan here.

IMPORTANT SAFETY INFORMATION (continued)

PATIENT COUNSELING INFORMATION

- Instruct the patient or caregiver to read the FDA-approved patient labeling, which includes information on the appropriate oral or feeding tube preparation, administration, and storage.

You may report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see full Prescribing Information for a complete discussion of the risks associated with DOJOLVI.

REFERENCES: 1. DOJOLVI Prescribing Information, October 2023. **2.** Knottnerus SJG, Bleeker JC, Wüst RCI, et al. *Rev Endocr Metab Disord*. 2018;19(1):93-106. **3.** Vockley J, Marsden D, McCracken E, et al. [published correction appears in *Mol Genet Metab*. 2015;116(3):221]. *Mol Genet Metab*. 2015;116(1-2):53-60. **4.** Fujihira K, Takahashi M, Wang C, Hayashi N. *Front Nutr*. 2023;10:1192223. **5.** Data on file. Ultragenyx Pharmaceutical Inc, November 2025.

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MRCP-UX007-01057 01/2026