June 30, 2020

Dear Patients and Families Living with LC-FAOD,

We are pleased to share the news today that the U.S. Food and Drug Administration (FDA) approved DOJOLVI™ (triheptanoin) oral liquid, the first and only medicine to treat long-chain fatty acid oxidation disorders (LC-FAOD).

This is a significant advancement for the LC-FAOD community and could not have happened without those individuals who participated in the triheptanoin clinical trials and clinical development efforts. We also recognize caregivers and family members who supported their participation, and the healthcare providers and researchers for their invaluable contributions.

We understand that the community may want to learn more about this news, and we have included below information for you. We value an open dialogue with patients and families and are interested in hearing any questions or comments you might have moving forward. Jessica Riviere and Kristin Voorhees from our Patient Advocacy team are always available to speak with the community. Please reach out to us at any time at patientadvocacy@ultragenyx.com.

Information about DOJOLVI and Patient Access to DOJOLVI

DOJOLVI is a prescription medicine used to treat long-chain fatty acid oxidation disorders (LC-FAOD) in children and adults.

IMPORTANT SAFETY INFORMATION

What are the possible side effects of DOJOLVI?

- **Feeding tube problems.** Feeding tubes may not work as well or stop working over time when taking DOJOLVI. **Do not use DOJOLVI in feeding tubes made of polyvinyl chloride (PVC),** a solid plastic material. Monitor the feeding tube to make sure it is working properly.
- **Intestinal absorption problems in patients with pancreatic insufficiency.** If you have pancreatic insufficiency, consult with your healthcare provider as it may affect how well DOJOLVI works.
- The most common side effects of DOJOLVI are:
    - stomach (abdominal) pain
    - diarrhea
    - vomiting
    - nausea

These are not all the possible side effects of DOJOLVI. Call your healthcare provider for medical advice about side effects. You may report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657 or FDA at 1-800-FDA-1088.

Before taking DOJOLVI, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if DOJOLVI will harm your unborn baby. **Pregnancy Safety Study:** There is a pregnancy safety study for women who take DOJOLVI during pregnancy. The purpose of this study is to collect information about your health and your baby's health. You can talk to your healthcare provider or contact 1-888-756-8657 to enroll in this study or get more information.
- are breastfeeding or plan to breastfeed. It is not known if DOJOLVI passes into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take DOJOLVI.
- are taking a pancreatic lipase inhibitor, such as orlistat, as it may affect how well DOJOLVI works.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

How should I take DOJOLVI?

- See the detailed **"Instructions for Use"** at the end of the Patient Information Leaflet for instructions about how to mix and take DOJOLVI by mouth in soft foods or drinks or how to mix and give DOJOLVI through feeding tubes.
- Take DOJOLVI exactly as your healthcare provider tells you.
- Your healthcare provider may start you on a low dose of DOJOLVI and slowly increase your dose to help avoid side effects. **If you are taking another medium chain triglyceride (MCT) product, stop taking the MCT before starting DOJOLVI**.

Please see full Prescribing Information, including the Patient Information Leaflet, for additional Important Safety Information.

Ultragenyx is committed to ensuring that any patient who could benefit from DOJOLVI has access to the medicine and that out of pocket costs are not a barrier to treatment.

We will offer a variety of services through UltraCare, our comprehensive, one-on-one, personalized support service that can help all patients who are prescribed DOJOLVI. UltraCare professionals will help patients understand their insurance coverage and find and navigate available financial assistance and patient assistance programs that can help to cover co-pay and other out-of-pocket costs. The UltraCare team will also work with patients, physicians and dietitians to provide support that is based on the recommendations, advice and prescription of healthcare providers and support patients' ongoing treatment adherence and needs through education and personalized counseling based on

orders from the healthcare provider. Patients and families may visit **UltraCareSupport.com** to learn more and connect with our dedicated team of specialists known as UltraCare Guides who are available Monday-Friday, 6:00am-5:00pm PT.

Our Ongoing Commitment to LC-FAOD

We are deeply committed to the patients and families affected by LC-FAOD and this extends beyond developing and providing access to DOJOLVI, including key areas such as:

- Diagnosis: Ultragenyx is sponsoring genetic testing for individuals known to have or suspected of having LC-FAOD and who do not already have a molecular confirmation in their medical record. Physicians must order the test and patients will not be responsible for the cost of the genetic test.

- Research: We will collect high-quality data through our LC-FAOD Disease Monitoring Program (DMP), a long-term prospective outcomes study. Adult and pediatric patients with LC-FAOD on any treatment (not just DOJOLVI) can participate. The DMP will study topics that patients and families have told us are priorities, such as better understanding disease progression, long-term complications of LC-FAOD, and the burden of LC-FAOD on patients and families. In addition, this study will evaluate the long-term outcomes of DOJOLVI treatment. Our goal is to establish a robust set of data that scientists and healthcare providers can use to increase our understanding of the disease and the role of DOJOLVI, as well as generate future research.

Thank you for your support and engagement. We look forward to continued collaboration.

Sincerely,

Camille L. Bedrosian, MD
Chief Medical Officer and Executive Vice President
Ultragenyx Pharmaceutical