CADTH

CADTH Reimbursement Review

Patient Input

TRIHEPTANOIN (Dojolvi)

(Ultragenyx Canada Inc)

Indication: Long-chain fatty acid oxidation disorders

CADTH received patient input from:
MitoAction

April 23, 2021

CADTH

CADTH Reimbursement Review
Patient Input Template

Name of the Drug and Indication	**Dojolvi**
Name of the Patient Group	**MitoAction**
Author of the Submission	[redacted]
Name of the Primary Contact for This Submission	[redacted]
Email	[redacted]
Telephone Number	[redacted]

1. About Your Patient Group

Describe the purpose of your organization. Include a link to your website.

MitoAction's mission is to improve the quality of life for children, adults, and families living with mitochondrial disease through support, education, outreach, advocacy, clinical research initiatives and by granting wishes for children affected by mitochondrial disease.

www.mitoaction.org

2. Information Gathering

CADTH is interested in hearing from a wide range of patients and caregivers in this patient input submission. Describe how you gathered the perspectives: for example, by interviews, focus groups, or survey; personal experience; or a combination of these. Where possible, include **when** the data were gathered; if data were gathered **in Canada** or elsewhere; demographics of the respondents; and **how many** patients, caregivers, and individuals with experience with the drug in review contributed insights. We will use this background to better understand the context of the perspectives shared.

MitoAction has engaged with the patient community through our weekly support calls, Facebook groups, Mito411 Support line and have received direct feedback from the U.S. patient community about their positive experience with Dojolvi. Since the US approval of Dojolvi, we have interacted with dozens of adult patients and parents who have shared their feedback

3. Disease Experience

CADTH involves clinical experts in every review to explain disease progression and treatment goals. Here we are interested in understanding the illness from a patient's perspective. Describe how the disease impacts patients' and caregivers' day-to-day life and quality of life. Are there any aspects of the illness that are more important to control than others?

Patients who have a Long Chain Fatty Acid Oxidation (LC-FAOD) disorder have trouble breaking down fat to produce usable energy. Symptoms can include lethargy, irritability, noticeably enlarged liver, weakened heart muscle, abnormal heart rhythms, total failure of the combine lung and heart function, poor muscle tone and periodic severe muscle pain caused by skeletal muscle breakdown (rhabdomyolysis) and cardiac failure, among others. Day-to-day, patients are required to manage a strict diet in order to manage fat intake and energy reserves. Patients often have to take breaks or naps during the day and activities that others might see as simple, take considerable effort and cause significant energy depletions in patients with LC-FAOD. Therefore, patients often can't participate in normal day-to-day activities as this become too draining and causes extreme exhaustion, which can lead to hospitalization and damage to their organs. As patients deplete their energy levels, this can significantly impact their organ function, lead to sever muscle weakness and because what is known as a "mito crash." So, a patient must manage their energy exertion throughout the day and even what might otherwise be seen as a simple task, can physically overwhelm a patient with LC-FAOD. Because patients must limit their activity, this can lead to depression, isolation and other mental health issues, which is very common with patients with a rare disease. LC-FAOD is a progressive disease, but with proper treatment and disease management, the hope is that patients can life full and meaningful lives, despite their diagnosis.

4. Experiences With Currently Available Treatments

CADTH examines the clinical benefit and cost-effectiveness of new drugs compared with currently available treatments. We can use this information to evaluate how well the drug under review might address gaps if current therapies fall short for patients and caregivers.

Describe how well patients and caregivers are managing their illnesses with currently available treatments (please specify treatments). Consider benefits seen, and side effects experienced and their management. Also consider any difficulties accessing treatment (cost, travel to clinic, time off work) and receiving treatment (swallowing pills, infusion lines).

Prior to the availability of Dojolvi, patients' only option was the use of over-the-counter MTC oils. These products are not regulated by the FDA, dosing and quality vary among manufactures and compliance is difficult to manage. These products also can be very costly for families. With the availability of Dojolvi, now patients can be managed by their clinician with a regulated therapy with controlled dosing and product quality. This treatment is readily accessible in the US with a prescription and due to the financial assistance programs, this ensures that Dojolvi is accessible to every patient who may benefit from the therapy.

5. Improved Outcomes

CADTH is interested in patients' views on what outcomes we should consider when evaluating new therapies. What improvements would patients and caregivers like to see in a new treatment that is not achieved in currently available treatments? How might daily life and quality of life for patients, caregivers, and families be different if the new treatment provided those desired improvements? What trade-offs do patients, families, and caregivers consider when choosing therapy?

Dojolvi provides a treatment that allows patients to properly break down fats and therefore produce energy to function. Patients report having energy to participate in life activities that prior to the treatment, they were not able to enjoy. The energy depletion for patients with LC-FAOD can be debilitating. The level of exhaustion is almost incomprehensible for someone who has never experienced this level of fatigue. A good analogy is to consider running your house on one AA battery that never charges being 20%. This is the type of energy depletion and fatigue someone with LC-FAOD experiences. Not to mention the devastation on organs and body functions. One particular high school student has shared that she was able to enjoy her senior year activities, and prior to treatment, she would not have been able to do. She even looks forward to the potential for her to literally walk, not use her wheelchair to walk at graduation. Other parents have shared that their children are able to participate in extracurricular activities, and perhaps may not require as much rest time during the day. Patients with LC-FAOD have to be very cognisant of their energy usage, often taking naps throughout the day in order to get through the day.

6. Experience With Drug Under Review

CADTH will carefully review the relevant scientific literature and clinical studies. We would like to hear from patients about their individual experiences with the new drug. This can help reviewers better understand how the drug under review meets the needs and preferences of patients, caregivers, and families.

How did patients have access to the drug under review (for example, clinical trials, private insurance)? Compared to any previous therapies patients have used, what were the benefits experienced? What were the disadvantages? How did the benefits and disadvantages impact the lives of patients, caregivers, and families? Consider side effects and if they were tolerated or how they were managed. Was the drug easier to use than previous therapies? If so, how? Are there subgroups of patients within this disease state for whom this drug is particularly helpful? In what ways? If applicable, please provide the sequencing of therapies that patients would have used prior to and after in relation to the new drug under review. Please also include a summary statement of the key values that are important to patients and caregivers with respect to the drug under review.

Dojolvi is available in the U.S. to patients with a prescription from a medical professional. Finally having an FDA approved treatment allowed the community to have a regulated therapy that could be managed by their medical team.

7. Companion Diagnostic Test

If the drug in review has a companion diagnostic, please comment. Companion diagnostics are laboratory tests that provide information essential for the safe and effective use of particular therapeutic drugs. They work by detecting specific biomarkers that predict more favourable responses to certain drugs. In practice, companion diagnostics can identify patients who are likely to benefit or experience harms from particular therapies, or monitor clinical responses to optimally guide treatment adjustments.

What are patient and caregiver experiences with the biomarker testing (companion diagnostic) associated with regarding the drug under review?

Consider:

- Access to testing: for example, proximity to testing facility, availability of appointment.
- Testing: for example, how was the test done? Did testing delay the treatment from beginning? Were there any adverse effects associated with testing?
- Cost of testing: Who paid for testing? If the cost was out of pocket, what was the impact of having to pay? Were there travel costs involved?
- How patients and caregivers feel about testing: for example, understanding why the test happened, coping with anxiety while waiting for the test result, uncertainty about making a decision given the test result.

8. Anything Else?

Is there anything else specifically related to this drug review that CADTH reviewers or the expert committee should know?

Dojolvi was the first FDA approved therapy for patients affected by LC-FAOD. This was monumental for this rare disease community to finally have an approved treatment that truly has shown a tremendous impact in their quality of life.

CADTH

Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH reimbursement review process, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.

No.

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.

No.

3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

Company	Check Appropriate Dollar Range			
	$0 to 5,000	$5,001 to 10,000	$10,001 to 50,000	In Excess of $50,000
Ultragenyx Pharmaceuticals			X	

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name: Kira Mann
Position:CEO
Patient Group: MitoAction
Date: 4/23/2021