ultragenyx pharmaceutical

LC-FAOD RESEARCH PROGRAMS

Research Opportunity:

LC-FAOD Disease Monitoring Program (DMP)

Participate in LC-FAOD research and help make an impact

Why are we doing this study?

Every participant in the DMP plays a key role in contributing to long-chain fatty acid oxidation disorders (LC-FAOD) research. The data generated by the study will be used to inform and support the entire LC-FAOD patient and care provider community. The overarching goal of the DMP is to better understand the disease—from the burden of disease on the people who live with it to the impact of disease management, including safety and effectiveness of treatment.

Who can participate?

Anyone diagnosed with an **LC-FAOD**, including **CPTI**, **CACT**, **CPTII**, **VLCAD**, **TFP** and **LCHAD**

What is the DMP?

- Largest, longest (about 10 years), and broadest LC-FAOD long-term observational study to date
- Global study to build on earlier clinical findings in larger, more diverse populations
- No intervention or treatment provided as part of the DMP

What is involved?

- Approximately 150 participants
- Approximately 18 sites the United States and Canada
- Involves visits to study sites and interactions with healthcare providers
- Support is provided for travel costs to clinic visits

For additional study information including site locations, scan the QR or visit https://clinicaltrials.gov/study/NCT04632953

The DMP is designed to better understand LC-FAOD

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Specific outcomes of interest include:

Frequency and duration of LC-FAOD–related major clinical events* and rates, and duration of hospitalizations

*Episodes of rhabdomyolysis, hypoglycemia and/or cardiomyopathy that require intervention in the emergency setting (ER/acute care) and/or result in hospitalization

How your disease may change over time

The DMP also addresses the FDA post-marketing requirement of a pregnancy safety study while on therapy

Impact of LC-FAOD on you and your family

The DMP will advance LC-FAOD science through data-sharing

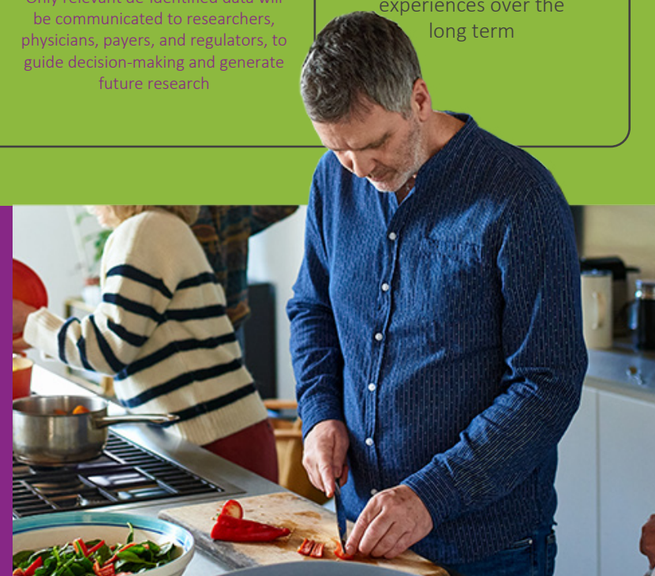
The DMP may be an important scientific and clinical resource for the LC-FAOD community, HCPs, and researchers

The DMP may provide benefits across the healthcare ecosystem, including to rare disease individuals with LC-FAOD, patient advocacy groups, HCPs, reimbursement authorities and researchers

As a DMP participant, you will be able to readily access your data, and privacy is paramount:

Only relevant de-identified data will be communicated to researchers, physicians, payers, and regulators, to guide decision-making and generate future research

The DMP will provide an opportunity for you to share with the scientific community and provide feedback to researchers about your experiences over the long term



You can help generate valuable information for the LC-FAOD community.
To learn more, talk to your healthcare provider or contact TrialRecruitment@Ultragenyx.com

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