

Ultragenyx's LC-FAOD Odyssey Study: From Patient Experience to Patient Empowerment

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MitoAction Monthly Expert Webinar Series



Introductions



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Today's Webinar

About Ultragenyx

- Our commitment to people living with LC-FAOD

How the LC-FAOD community is helping to advance research at Ultragenyx

Overview of the LC-FAOD Odyssey study

- Key study details
- Live Demo: how to enroll and access the PicnicHealth timeline, a visual of your medical history
- How you can participate

Q&A

About Ultragenyx

Ultragenyx is a **biopharmaceutical company** committed to bringing patients novel products for the treatment of **rare and ultra-rare diseases, including LC-FAOD**

Headquartered in **Novato, CA** with US offices in San Francisco Bay Area and Massachusetts, and global offices in Europe and Latin America, and a presence in Canada

Founded by Emil Kakkis, MD, PhD in 2010 who began his work developing an enzyme replacement therapy for MPS I in partnership with The Ryan Foundation

More than **900 employees**



Patient Advocacy and Patient Engagement at Ultragenyx

OUR PURPOSE

Advance global rare disease advocacy through inclusive patient engagement and partnership.



Learn From People Living With Rare Diseases and Partner With Organizations

- Build **long-term relationships** with patient organizations
- **Understand experiences, challenges and unmet needs** of patients and families
- Focus on **ensuring diverse voices and perspectives**



Incorporate Community Perspectives Into Our Programs and Decisions

- Collaborate with cross-functional teams at Ultragenyx to:
 - **Establish framework for how patient and caregiver input is consistently incorporated** at all stages of development
 - Transparently **share with external communities the impact of their input**



Address the Unmet Needs of Rare Communities

- **Support the vital work** of patient organizations
- Develop **education, resources, and programs** that meet the needs of patient communities
- Provide information and engage with the rare disease community at [UltraRareAdvocacy.com](https://ultrarareadvocacy.com) and on **Facebook**

Ultragenyx and the Community: Partners in Advancing Research

Ultragenyx is committed to:

- **Advancing research** to better understand the science/treatment of LC-FAOD since 2013
- **Engaging with patients**, caregivers, and family members as partners across the continuum of LC-FAOD interventional and observational research
- **Supporting the research** interests of scientists and clinicians who are focused on globally generating evidence to improve our understanding of LC-FAOD and its disease management

This commitment includes:



Fostering partnerships to elevate the patient voice



Uncovering and addressing unmet patient needs



Empowering patients to successfully navigate the healthcare system



Developing research programs based on patient and caregiver insights

People Living with LC-FAOD Shape Ultragenyx's Research Goals



Ultragenyx convenes forums such as the LC-FAOD Patient Leadership Council and advisory boards to generate insights that aim to guide company decision-making

- Understand what is most important to people living with LC-FAOD
- Allow for open and transparent dialogue that helps determine appropriate ways to support the community

LC-FAOD Patient Leadership Council (PLC)

The PLC, established in 2017, aims to provide a bridge between the LC-FAOD community and the company, to support open and transparent dialogue, and to determine appropriate avenues for partnership and ways to support the community

Patient and caregiver advisory boards

Ultragenyx has held five advisory boards between 2015 - 2021 to generate insights that help inform strategy, planning, and decision-making, from research and development to commercialization

The LC-FAOD Odyssey Study Aims to Address Challenges and Research Questions of Critical Importance to the Patient Community

Improve difficulties in navigating the healthcare system with a chronic, rare condition

Understand natural history of how LC-FAOD progresses and may change over time

Impact of LC-FAOD on a person's ability to perform some daily living activities

Understand differences among the 6 types of LC-FAOD

Insights from 2015–2019 informed Ultragenyx's decision to launch the LC-FAOD Odyssey study in 2020

LC-FAOD Odyssey:

An Opportunity for Patients and Parents to Help Advance LC-FAOD Research

21 May 2021

Eliza Kruger, Director, Global HEOR

Ultragenyx Pharmaceutical Ltd



Agenda

LC-FAOD Odyssey Study Overview

- Purpose
- Overview of PicnicHealth
- Key Study Details

Live Demo

- How to Sign Up
- How to Use the PicnicHealth Timeline

Next Steps and Q&A

What is LC-FAOD Odyssey?

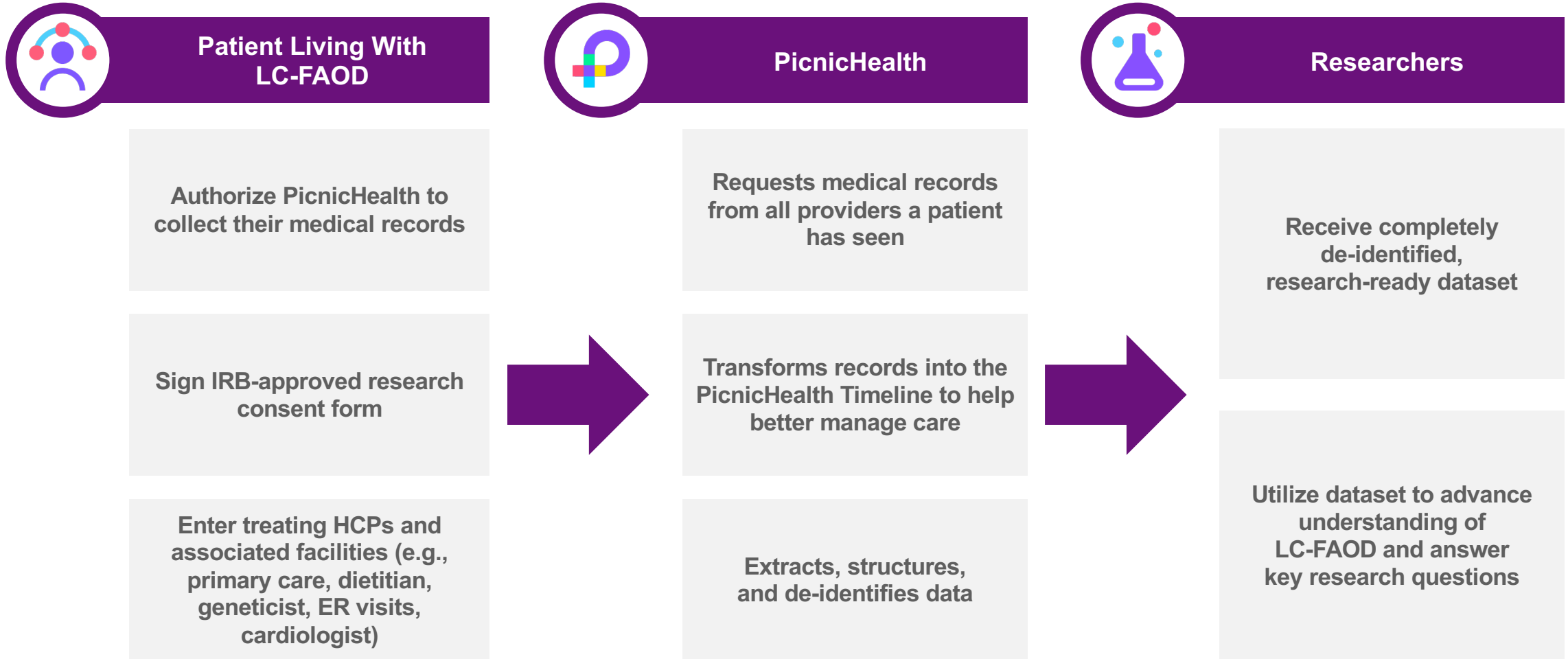
What are we doing?

- **LC-FAOD Odyssey is a central IRB-approved research study from Ultragenyx and PicnicHealth,** a digital health company, to centralize medical records for patients and create an anonymized dataset for researchers to better understand LC-FAOD
- The study is **currently enrolling both adults and children who are living with LC-FAOD in the U.S.** and can be used by patients/caregivers to better manage their care

Why are we doing it?

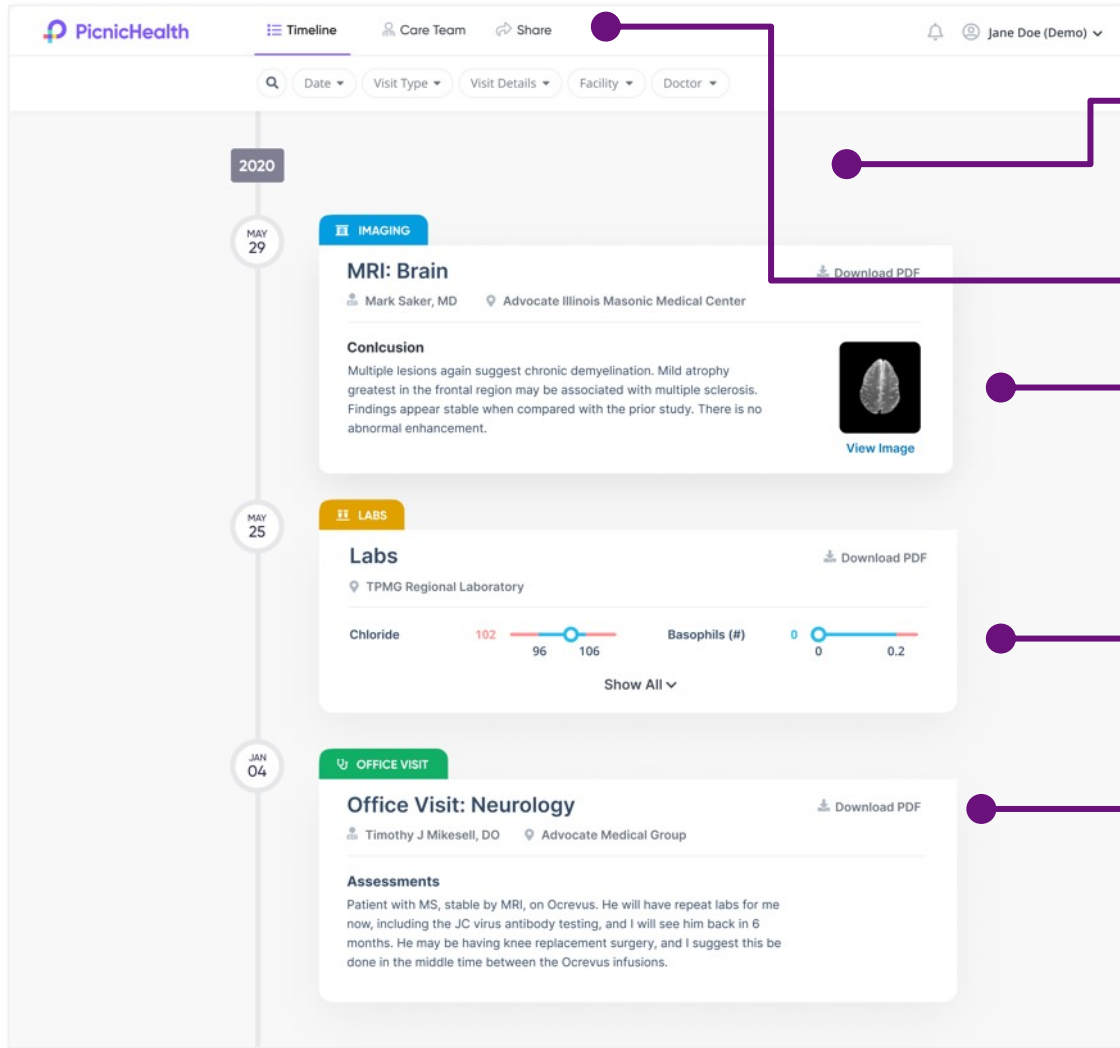
- **LC-FAOD Community:** Better understand LC-FAOD progression, how it is managed in the real world, and the effectiveness of existing treatments
- **Patients:** Centralize medical records to help patients access and organize their medical records, better manage their care, and contribute their anonymized data to research
- **Physicians and Researchers:** Construct an anonymous, comprehensive LC-FAOD dataset that can be used by academic researchers
- **Ultragenyx:** Better understand LC-FAOD progression and disease management, use and effectiveness of treatments, and further the commitment to advancing LC-FAOD research

How does this work?



What is the PicnicHealth Timeline?

The PicnicHealth Timeline



Access all medical records in one place

Share records with family and physicians

View images through PicnicHealth's FDA-approved and HIPAA-compliant image viewer

Track all labs longitudinally

Download original source records

How does PicnicHealth protect and anonymize patient data?

The PicnicHealth team are patients too.

PicnicHealth knows how important privacy and security are. That is why they use stringent deidentification protocols, have HITRUST certification, use military grade 256-bit encryption, are HIPAA compliant, use an external IRB, and require active patient consent.



HIPAA compliant.

PicnicHealth follows the highest standards set by law.



End-to-end encryption.

PicnicHealth uses the highest cryptographic standards available.



Your data, under your control.

PicnicHealth never shares your personal information without your explicit permission



There for you.

If in doubt, ask. PicnicHealth is there to make sure you fully understand their practices.

Key study details

Who can join?

Those living with any type of LC-FAOD who live in the U.S., including caregivers of children.

What are we asking of you?

We ask that you visit the study website for more information. There you can review the study, consent to PicnicHealth requesting records on your behalf, and complete surveys during the study.

What will I get from this study?

1. Get access to all your medical records and be able to contribute anonymized data to research.
2. You will receive up to \$150 in the first year for enrolling and taking surveys.
3. Participating will not affect your ability to enroll in clinical trials.

What will the LC-FAOD research community get?

LC-FAOD researchers will get access to an anonymized, real-world dataset to help future LC-FAOD research.

The banner features the logos for ultragenyx pharmaceutical and PicnicHealth at the top left. A 'Join Free' button is located at the top right. The main title is 'LC-FAOD Odyssey' in large blue font, with the tagline 'Turning your experience with rare disease into meaningful evidence.' below it. The primary message is 'Simplify your medical records and contribute to LC-FAOD research' in bold black text. To the right is a photograph of a family (a man, a woman, and a child) in a warm embrace. Below the main text is a paragraph explaining the partnership between Ultragenyx and PicnicHealth to conduct research on LC-FAOD, aiming to understand its real-world impact and improve future care through patient experience data.

ultragenyx pharmaceutical | PicnicHealth

Join Free

LC-FAOD Odyssey

Turning your experience with rare disease into meaningful evidence.

Simplify your medical records and contribute to LC-FAOD research

Ultragenyx and PicnicHealth are partnering to conduct innovative research that aims to better understand how LC-FAOD (Long-chain Fatty Acid Oxidation Disorders) affect real people like you. Understanding how a disease is managed and treated and the effects of these approaches in the real-world, combined with understanding your personal experience based on your answers to questionnaires, can help address some important questions that could improve the future quality of care.

Visit the study website to learn more!
<https://picnichealth.com/lcfaod>

LIVE DEMO: Visit the LC-FAOD website to get started!



LC-FAOD Odyssey

Turning your experience with rare disease into meaningful evidence.

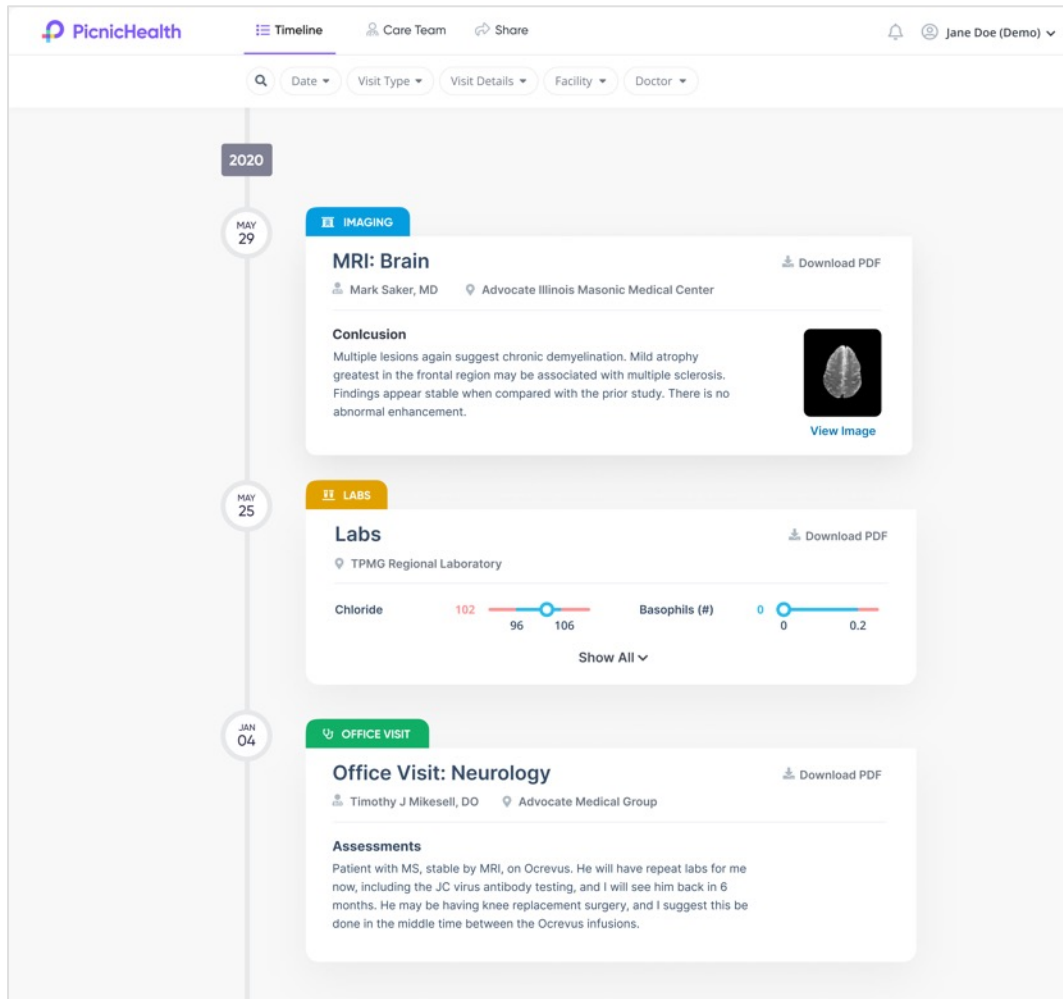
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Visit the study website at
<https://picnichealth.com/lcfaod>

Once you have signed up and PicnicHealth has collected your records, you'll get your own PicnicHealth Timeline to view your full health history!



Visit the demo PicnicHealth Timeline at <https://demo.picnichealth.com>

How you can participate in/help with LC-FAOD Odyssey

1 Visit the study website and sign up if you choose to



<https://picnichealth.com/lcfaod/>

2 Share information about LC-FAOD Odyssey with your communities



Feel free to share the study website and information about the study with your communities if you choose to

3 Ask any questions you have



Reach out to PicnicHealth (faodstudy@picnichealth.com) with any questions you have about the study

LC-FAOD Odyssey Status Update

Enrollment

- 24 patients/caregivers of patients living with LC-FAOD have enrolled since the study launched in August 2020

Preliminary results (13 patients)

- PicnicHealth has collected ~7.5 years of data on average for each participant and has retrieved records quickly (~4 weeks on average)

Coming soon: Community Representative Program

- Individuals with LC-FAOD who are enrolled in the Odyssey study will be invited to share their experience with LC-FAOD Odyssey and PicnicHealth

Research Will Be Shared With Participants

- Once researchers analyze anonymized and aggregated data and publish findings, we will be sure to share this with those who sign up—we're in this together!

Any questions?



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Frequently asked questions

1

Who can join this study?



People of all ages who have been diagnosed with LC-FAOD, live in the U.S., and have received medical care in the U.S. in the past 7 years can join. An additional form to consent to the study is required for participants 7 to 17 years old.

2

What data will be collected? How is patient privacy protected?



PicnicHealth will collect medical records from all doctors, hospitals, and clinics that you tell us about. We abide by HIPAA, use 256-bit SSL encryption, and will never share your data without your explicit permission.

3

How long will it take to get my or my child's medical records?



It takes about 4 weeks to retrieve most of your historical medical records. After that, it can take another couple of months for us to track down the oldest and trickiest records to access. The timing depends on how quickly your doctors respond to PicnicHealth's requests for your records.

4

Can I stop participating in the study?



Yes. Your participation in this study is completely voluntary and you can withdraw at any time. If you choose to stop participating, your medical records will remain available for you through your PicnicHealth account. At that time, we will stop collecting any new medical records.

Thank You!



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