Informed Consent Form

Agreement to be in the LC-FAOD Odyssey Study

Title of Study:	"LC-FAOD Odyssey: Research study collecting longitudinal data of patients with long-chain fatty acid oxidation disorders"
Protocol Study:	C-FAO-001
Sponsor:	Ultragenyx Pharmaceuticals, Inc.
Person in Charge of the Research Study (Investigator):	Noga Leviner
Telephone number(s), Daytime & After Hours:	(415) 801-0572

Introduction

You (“you” refers to you or your child throughout this consent form) are invited to take part in research for long-chain fatty acid oxidation disorders (LC-FAOD) using your medical data. Because you have one of the six LC-FAOD types (CPT I, CACT, CPT II, VLCAD, TFP, or LCHAD), you can help advance research to better understand your disease. This document describes the purpose of this research study and how your data will be used, so that you can make an informed decision about joining the study. Please take your time to read the following information carefully.

The investigator is being paid by the sponsor (the company paying for this study) to conduct this research study.

What is the purpose of this research study?

At PicnicHealth, we believe that every patient should have control and a complete view of their medical data. In the hands of researchers, this data can be useful to better understand the disease and improve patient care.

The purpose of LC-FAOD Odyssey is to help Ultragenyx better understand these diseases so that they can improve how they are managed. Some LC-FAOD research objectives your health information can help answer include understanding:

- The frequency and impact of metabolic crises, such as hospitalizations
- The extent and impact of avoiding everyday activities, including physical activities
- The emotional and social toll managing the disease can take (for patients & caregivers)
- Associated symptoms, health complications, and other disease-related conditions
- Disease progression
- Treatment patterns and their effectiveness
- Differences in outcomes by LC-FAOD type

Your de-identified data may be shared with select researchers outside of Ultragenyx. This data-access program is designed to ensure that the data extracted through this Ultragenyx-sponsored study is also made available to qualified researchers working to advance LC-FAOD research for the public good. Researchers interested in studying LC-FAOD using the de-

identified data collected from this PicnicHealth and Ultragenyx study can submit an application to PicnicHealth. PicnicHealth then follows a process to evaluate potential partners based on the validity of the research questions and commitment to advance science for the public good.

Who is conducting this research?

The study is under the direction of PicnicHealth, a digital health company that helps patients collect & manage their medical records and support research. Ultragenyx, a biopharmaceutical company that does scientific research to develop new treatments for a range of rare medical conditions, including LC-FAOD. Ultragenyx is providing financial support to PicnicHealth to cover the cost of retrieving medical data for this study.

How many people will participate in this study?

Up to 200 boys, girls, men, and women with LC-FAOD may participate in this study.

How much time will this study require of me?

Time will be required to set up an account, review this document, and answer basic research questionnaires that should take approximately 10 minutes. You will be asked to answer a research questionnaire up to four times a year, each questionnaire should not take more than 10 minutes.

What will happen if I join the study?

If you agree to join you'll be asked to:

- List information about your healthcare providers (doctors, hospitals, and/or clinics)
- Authorize PicnicHealth to collect your medical records on your behalf
- Answer questionnaires to better understand how you're feeling and your experiences

You will receive a subscription to PicnicHealth - an online service that helps patients collect and manage their medical records - for free as long as you participate in the study. Neither you nor your insurance company will be billed for PicnicHealth's services. As part of these services, PicnicHealth will:

- Collect your medical records on your behalf from the healthcare providers that you authorize
- Convert your medical records so that you can access them online through your PicnicHealth timeline
- Collect your ongoing medical records on your behalf from the doctors who continue to provide care for you

Once the de-identified information from your medical records have been shared with PicnicHealth's research partners, the de-identified medical information may no longer be protected by federal privacy law and may be subject to re-disclosure. PicnicHealth may contact you in the future with new information about this study. Additionally, PicnicHealth may contact you about other similar IRB-approved studies like this one in the future to determine if you would like to participate.

Data collected from this study will be shared with Ultragenyx and its authorized representatives and may be made available to qualified researchers working to advance long-chain fatty acid oxidation disorders research. Except for those circumstances described below, PicnicHealth takes steps to remove personally identifying information, such as your name, picture, and/or telephone number, and assigns a unique patient identification number before your data is used and shared for research purposes. We'll refer to data, with your personally identifying information removed, as "de-identified data."

Your de-identified health information and data will be analyzed by Ultragenyx in order to better understand LC-FAOD. The study data may be recorded, analyzed, and published. You will not receive any compensation or share in any financial benefits from products, tests or discoveries that result from this research. No names or personally identifying information will be disclosed to any researchers or research organizations. No patient will be identified in any report or publication from this study.

PicnicHealth may partner with other research institutions, including government or academic organizations. Data access for these institutions will be controlled through an application process managed by PicnicHealth, where partners will be evaluated based on their commitment to advance science for the public good. PicnicHealth will grant free access for non-commercial research - meaning, research that is not intended to make money but rather to contribute to science and the public good.

You may at any point contact PicnicHealth to get more information about how your de-identified data is being used for research purposes.

What data will be collected?

This study will look at medical data that has already been recorded in your medical records during your normal medical care. You will not need to make any special doctors' visits as part of the study. The following information will be extracted from your medical records when available:

- Demographics (gender, date of birth, etc.)
- Visits to healthcare providers (date, care site, etc.)
- Data about LC-FAOD (diagnosis date, type, disease management protocol, treatments, diet, etc.)
- Medical history (other diseases you may have or have had)
- Vital signs (heart rate, body temperature, etc.)
- Results of physical examinations
- Blood tests and other laboratory tests
- Procedures (imaging, surgeries, etc.)
- Medications
- Doctor's notes

The study will also use online questionnaires to collect important information that may not exist in your medical records, such as how you're feeling, up to four times per year.

What type of analysis will be done with my data?

Some research objectives that your data may help answer could include but are not limited to:

- Understanding challenges patients face, including the frequency and impact of metabolic crises
- Understanding LC-FAOD disease progression
- Understanding differences in outcomes between LC-FAOD types
- Understanding effectiveness in treatments and disease management approaches

It's hard for us to know every research question that your data may help answer. Since science is continually evolving, new questions may arise that we don't know yet.

Additionally, we may link the data collected here to other existing healthcare data sets (e.g., patient registries, claims) to help answer key research questions. Any data you contribute will be de-identified before using it in any study regardless of whether it is linked to other healthcare data sets. We take the same steps described above to de-identify combined healthcare data sets before they are used or shared for research purposes.

How will my personal and health information be kept private?

As part of this study, data will be extracted from your medical records in a way to ensure that your medical and personal information is kept confidential. Your medical and personal information and copies of your medical records will be held by PicnicHealth.

You will be assigned a unique patient identification number. The medical data used for this study will be labeled with this identification number; the data will not be labeled with your name, picture, or any other personally identifying information.

Ultragenyx and its authorized representatives may collect and process this information for purposes of carrying out and evaluating the study, developing and making regulatory submissions, and meeting legal and regulatory obligations (for example, in relation to the safety of Ultragenyx products).

Applicable national laws and regulations to protect the personalized data in your medical records will be strictly followed. The confidentiality of your personal information will be protected throughout the study and afterwards. It will be kept confidential; your medical and personal information may be revealed only if required by law. National health authorities, regulatory bodies, or other overseeing agencies and Ultragenyx and/or its authorized representatives may want to examine your data within the scope of an inspection according to national law to ensure that this study is done properly. If the study results are presented at meetings or printed in publications, your name will not be used.

In certain situations, to make sure that the study is being done properly or to check the quality of the data, the following people and groups of people might be granted direct access to copies of your medical records (i.e. they may see your medical and personal information) without violating the confidentiality of your data:

- The investigator
- PicnicHealth personnel directly involved with the medical data retrieval processes
- If applicable, study auditors from organizations who have had access to the database
- The Institutional Review Board responsible for protecting the rights and safety of the patients who take part in research studies
- Regulatory health authorities (government agencies involved in keeping research safe for people)

The Institutional Review Board (IRB), IntegReview, and accrediting agencies may inspect and copy your records, which may have your name on them. Therefore, total confidentiality cannot be guaranteed.

You have the right to see and get a copy of your medical records kept by PicnicHealth. Your authorization (permission) to use and disclose (share) your health information does not have an expiration date, but that use and sharing will only be for the purposes described in this consent form.

You are free at any time to limit PicnicHealth's use and sharing of your health information, without penalty or other consequence. However, you may not be allowed to take part or continue to take part in this study if at any time you choose to limit PicnicHealth's use and sharing of your health information that is necessary for the completion of this study. As a result, you may no longer receive future updates to your PicnicHealth timeline.

You may change your mind and revoke (take back) this authorization at any time by writing to PicnicHealth at faodstudy@picnichealth.com. If you revoke this authorization, no additional health information about you will be retrieved nor shared. However, any data that has already been shared with a partner, up until the time you notify PicnicHealth to revoke your authorization, cannot be modified or deleted. This data will be de-identified and will not include personal information like your name or date of birth.

Are there possible benefits to agreeing that my personal and health data can be used in the study?

There is no direct medical benefit to you from being in this study. The information gained from this study may help scientists and doctors learn more about LC-FAOD. You and other patients with LC-FAOD or similar conditions could benefit from the results of this study in the future.

You will benefit from free access to PicnicHealth's medical record collection and management services as long as you participate in this study. With these services, you will be able to view your medical records data (e.g., lab results, prescriptions, and imaging files), download copies of records, and share your records with your doctors.

Are there any possible risks if I agree that my personal and health data can be used in the study?

There are no physical risks from participating in this study, but there may be privacy and confidentiality risks. Despite making our best efforts, it's never possible to fully guarantee that your personally identifiable information (e.g., name, date of birth) will never become known. For example, data may be vulnerable in transit or servers may be accessed by unauthorized individuals. Because study records have been labeled with a unique patient identification number instead of your name, picture, or other personally identifiable information, the risk of a breach of confidentiality is reduced, but there is still a chance someone could trace your records back to you because your records contain LC-FAOD information, a rare disease.

What alternative do I have if I do not want my data to be used in the study?

Your alternative is not to participate in this study. If you choose to not give consent and not be part of this study, you will not receive PicnicHealth's services free of charge. You can always choose to pay to use PicnicHealth's medical record collection and management services, independent of the study. If you use PicnicHealth independent of the study, you can authorize PicnicHealth to collect your medical records for your personal use and care coordination.

To learn more about PicnicHealth's services, you can visit https://picnichealth.com/.

Can I withdraw from the study?

Your participation in this research study is completely voluntary and you can withdraw at any time.

If you choose to stop participating in the study, you can continue to access your existing medical records through your PicnicHealth account but no new records will be added unless you choose to subscribe to the PicnicHealth service at your own expense.

You can request that your data be deleted from the PicnicHealth service at the time you withdraw from the study, or at any time before or after. However, you are not able to modify or delete the de-identified data that already has been shared with research partners.

Will I receive the results of this study?

We believe it's important for you to see the results of research produced using your data and we'll make reasonable efforts to share published results (e.g., research publications, conference abstracts) with you. Research takes time, so we appreciate your patients while scientists and clinicians are working on learning new things about LC-FAOD.

Who is financing this research?

This research study is being financed by PicnicHealth and Ultragenyx Pharmaceuticals Inc. and conducted by PicnicHealth.

Who can answer my questions about this study?

You can contact PicnicHealth for any questions.

Email:	faodstudy@picnichealth.com
Phone:	1-415-801-0572

If you do not want to talk to PicnicHealth, or if you have concerns or complaints about the research, or if you want to ask about your rights, you may contact IntegReview. IntegReview is a group of people that has reviewed this research study. The main goal of this review is to protect the rights and well-being of the human subjects participating in research studies. IntegReview's policy indicates that all concerns/complaints are to be submitted in writing for review to:

Email and Mailing Address:
integreview@integreview.com
Chairperson
IntegReview IRB

3815 S. Capital of Texas Highway
Suite 320
Austin, Texas 78704

If you are unable to provide your concerns/complaints in writing or if this is an emergency situation regarding subject safety, contact IntegReview's office at:

Phone: 1-512-326-3001 or toll free at 1-877-562-1589 (between 8 a.m. and 5 p.m. Central Time)

IntegReview has approved the information in this consent form and has given approval for the investigator to do the study. This does not mean IntegReview has approved your being in the study. You must consider the information in this consent form for yourself and decide if you want to be in this study.

Payment for being in the study

You will not be paid for being in this study.

Volunteering to be in the study

It is your choice if you want to be in the study. No one can force you to be in the study. You may not want to be in this study or you may leave the study at any time without penalty or loss of benefits to which you are otherwise entitled. If you want to stop participating in this study, you should email PicnicHealth at faodstudy@picnichealth.com

PicnicHealth or IntegReview may take you out of the study without your permission, at any time, for the following reasons:

- If you do not meet the study's eligibility criteria
- If you do not follow the investigator's instructions
- If the study is stopped

If you leave the study, no more information about you will be collected for this study. However, all of the information you gave us before you left the study will still be used.

SUBJECT'S BILL OF RIGHTS

You will be given a separate copy of the California Experimental Research Subject's Bill of Rights. If you have not received a copy of this document, please notify study staff.

Legal Rights

You will not lose any of your legal rights by signing this consent form.

I understand that I will be able to access an electronic copy of this entire form. I have read it, or it has been read to me. I understand the information and have had my questions answered. I voluntarily agree to the use of my personal and health data for this observational study as described above and authorize PicnicHealth to disclose (share) my health information as described in this Informed Consent Form.

You will be given a copy of this consent form.

Signature of Individual (or Legal Representative)

undefined

Printed Name

Date

Authority of Legal Representative: