UNIVERSITY OF CALIFORNIA, IRVINE
CONSENT TO ACT AS A HUMAN RESEARCH SUBJECT

Concept Elicitation Interviews to Understand Neuropathic Pain in Fabry Disease, and Cognitive Debriefing and Usability Testing of an Electronic Diary Containing Multiple Measures in Fabry Disease

You are being asked to participate in a research study. Participation is completely voluntary. Please read the information below and ask questions about anything that you do not understand. A researcher listed below will be available to answer your questions.

RESEARCH TEAM
Lead Researcher
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STUDY LOCATION:
University of California, Irvine ICTS (Institute for Clinical and Translational Science), Irvine, CA

STUDY SPONSOR:
Actelion Pharmaceuticals

WHY IS THIS RESEARCH STUDY BEING DONE?
The purpose of this research study is to conduct survey through phone and in-person interviews with subjects who have Fabry Disease (FD) The goal of this research study is to better understand the type of pain FD patients may experience, and to determine if an electronic diary containing various questionnaires may be used in a clinical trial investigating a new medicine for FD. This study does not involve drugs in any way.

Your input, together with the input from other subjects in this research study will help us to understand if the electronic diary is clear and understandable, accurately captures the experience patients with FD may have, and is easy enough to be utilized by patients.

HOW MANY PEOPLE WILL TAKE PART IN THIS STUDY?
Approximately 15 participants will take part in the research at UCI.

AM I ELIGIBLE TO PARTICIPATE IN THIS STUDY?
Please note this may not be a complete list of eligibility criteria. We have included a few examples of study criteria to help you better understand how your eligibility in the study will be determined; your study

team will go through the study eligibility criteria with you to verify if you qualify for participation in this study.

Inclusion Requirements
You can participate in this study if you:

- Are fluent in US English (i.e., able to speak, read, write and comprehend);
- Have read, understood and signed this written Informed Consent Form and the Health Insurance Portability and Accountability Act Authorization (HIPAA) form;
- Are at least 18 years of age or older;
- Have been diagnosed with Fabry disease with known genetic test results (i.e., presence of at least one GLA gene mutation) documented in your medical records;
- Have had Fabry-associated neuropathic pain in the last 3 months.

Exclusion Requirements
You cannot participate in this study if you:

- Have another pain condition that may interfere with the accurate understanding of pain associated with Fabry disease as determined by Dr. Kimonis;
- Have another gastrointestinal disease that may interfere with the accurate understanding of gastrointestinal symptoms associated with Fabry disease as determined by Dr. Kimonis;
- Have any other condition, in the opinion of Dr. Kimonis that would interfere with your ability to provide informed consent and successfully participate in the interview.

HOW LONG WILL THE STUDY GO ON?
This study include up to three sessions, one for obtaining your consent, one 30-60 minute telephone interview, then one in person interview visit (if selected). All fifteen subjects will be consented in person or via phone, and all will complete a 30 to 60 minute telephone interview. Ten out of the fifteen subjects will be selected to have an additional in-person interview that will take 60 to 90 minutes, approximately 2-6 weeks after the initial phone interview.

WHAT PROCEDURES ARE INVOLVED WITH THIS STUDY?
Before you can participate in the main part of the study...
You will have “screening” performed for this study to determine eligibility. The screening process helps the researchers decide if you meet the study requirements listed below. The screening procedures do not include any physical exams, tests or procedures. As part of the screening you will be discussing your neuropathic pain in the past 3 months with Dr. Kimonis.

During the main part of the study...
If the screening shows that you are eligible for the study and you choose to take part, then you will have complete the following:

- To learn about the study and provide consent for the study you will either meet Dr. Kimonis in person or discuss the study over the phone. The consent form will be mailed or emailed to you for your signature and returned to Dr. Kimonis. You will also have your eligibility verified;
- The study team will then schedule you to participate in a 30 to 60 minute telephone interview in which you will be asked questions about your symptoms with Fabry Disease.
- Approximately 2-6 weeks after the telephone interview, a subset of ten patients will be selected for an in depth 60 to 90 minute in-person interview Hewitt Hall on the UC Irvine campus, in which you will complete questionnaires on an electronic device. Then you will be asked questions about your experience completing the questionnaires.

	Visit 1 (in person or via mail)	Visit 2	Visit 3	Post Study
Consent (30-60 minutes)	**X**			
Telephone Interview (30-60 minutes)		**X**		
In person Interview (Only 10 of the 15 subjects will be selected) (60-90 minute)			**X**	
Demographic form (10 minutes)				**X**

After you complete the main part of the study (i.e., interviews)...
You will complete a brief demographic form and be compensated for your time. There are no follow-up visits or tests or questionnaires after the in-person interview (if you are selected for the in-person interview; if you are not selected for the in-person interview, you will have no additional visits after the telephone interview).

WHAT ARE THE POSSIBLE SIDE EFFECTS OR RISKS RELATED TO THE STUDY?
There is no risk of side effects or physical harm related to participating in this study since the study involves no drugs or treatment for your condition. The risks for this study are the possibility of feeling uncomfortable as you share your experiences with Fabry Disease and the potential for loss of confidentiality of your responses.

ARE THERE BENEFITS TO PARTICIPATING IN THIS STUDY?
Participant Benefits
You will not directly benefit from participation in this study.

Benefits to Others or Society
Your participation in the study may lead to a better understanding about FD. The information you provide may benefit others with FD in the future.

WHAT OTHER CHOICES DO I HAVE IF I DON'T WANT TO PARTICIPATE?
The alternative is not to participate in this study.

WILL I BE PAID FOR TAKING PART IN THIS STUDY?
Compensation
You will be paid $100 for the telephone interview, and potentially another $150 for the in-person interview, if you are selected. Total compensation for participation in the entire study is up to $250. If

you decide to withdraw from the study or are withdrawn by the research team, you will receive compensation for the visits and/or procedures that you have completed.

A $100.00 gift card (i.e., Visa gift card that can be used anywhere a credit card is accepted) will be mailed to you after completion of the telephone interview. If you are selected to participate in the in-person interview and you complete the in-person interview, you will receive an additional $150.00 gift card immediately after the interview.

Reimbursement
You will not be reimbursed for any out of pocket expenses, such as parking or transportation fees.

WHAT ARE THE COSTS OF TAKING PART IN THIS STUDY?
There is no cost to you for participation in this study. However there may be out-of-pocket expenses such as parking and transportation fees.

WHAT HAPPENS IF I AM INJURED BECAUSE I TOOK PART IN THIS STUDY?
Since there is no drug involved in this research study, we do not anticipate there being any participant injury. However, please follow the directions in the next two paragraphs in the unlikely case that you are injured.

It is important that you promptly tell the researchers if you believe that you have been injured because of taking part in this study. You can tell the researcher in person or call him/her at the number listed at the top of this form.

If you are injured as a result of being in this study, UCI will provide necessary medical treatment. The costs of the treatment may be covered by the University of California or the study sponsor, Actelion, or billed to you or your insurer just like other medical costs, depending on a number of factors. The University and the study sponsor do not normally provide any other form of compensation for injury. For more information about this, you may call UCI Human Research Protections (949) 824-6068 or (949) 824-2125 or by e-mail at IRB@research.uci.edu

WHAT HAPPENS IF I WANT TO STOP TAKING PART IN THIS STUDY?
You are free to withdraw from this study at any time. **If you decide to withdraw from this study, you should notify the research team immediately**. The research team may also end your participation in this study if you do not follow instructions, miss scheduled visits or keep appointments, the study sponsor decides to stop the study or your safety and welfare are at risk.

If you elect to withdraw or are withdrawn from this research study, you may choose to terminate the continued use or disclosure of your protected health information (PHI) for research purposes. The request to end the use or disclosure of your PHI should be made in writing.

HOW WILL INFORMATION ABOUT ME AND MY PARTICIPATION BE KEPT?
Subject Identifiable Data
All identifiable information collected about you will be removed and replaced with a code. A list linking the code and your identifiable information will be kept separate from the research data.

Data Storage
Research data will be maintained in paper format in a secure location at UCI. Only authorized individuals will have access to it.

Research data will also be stored electronically on a secure network in an encrypted file.

The audio recordings will also be stored in a secure location and transcribed. The recordings will be retained with the other research data. Transcripts will have all identifying information removed.

Data Retention
The researchers intend to keep the research data indefinitely. Other researchers may have access to the data for future research. Any data shared with other researchers, will not include your name or other personal identifying information.

WHO WILL HAVE ACCESS TO MY STUDY DATA?
The research team, authorized UCI personnel, the study sponsor, Actelion, and regulatory entities such as the Food and Drug Administration (FDA) and the Office of Human Research Protections (OHRP), may have access to your study records to protect your safety and welfare.

Any information derived from this research project that personally identifies you will not be released or disclosed by these entities without your separate written consent, except as specifically required by law. Research records provided to authorized, non-UCI entities will not contain identifiable information about you. Publications and/or presentations resulting from this study will not include identifiable information about you.

While the research team will make every effort to keep your personal information confidential, it is possible that an unauthorized person might see it. We cannot guarantee total privacy.

Investigator Financial Conflict of Interest
No one on the study team has a disclosable financial interest related to this research project.

WHO CAN ANSWER MY QUESTIONS ABOUT THE STUDY?
If you have any comments, concerns, or questions regarding the conduct of this research, please contact the research team listed at the top of this form.

If you wish to ask questions about the study or your rights as a research participant to someone other than the researchers or if you wish to voice any suggestions, problems or concerns you may have about the study, please contact UCI's Office of Research by phone, (949) 824-6068 or (949) 824-2125, by e-mail at IRB@research.uci.edu or at 141 Innovation Drive, Suite 250, Irvine, CA 92697.

What is an IRB? An Institutional Review Board (IRB) is a committee made up of scientists and non-scientists. The IRB's role is to protect the rights and welfare of human subjects involved in research. The IRB also assures that the research complies with applicable regulations, laws, and institutional policies.

HOW DO I AGREE TO PARTICIPATE IN THIS STUDY?
You should not sign and date this consent form until all of your questions about this study have been answered by a member of the research team listed at the top of this form. You will be given a copy of this signed and dated consent form, and the attached "Experimental Subject's Bill of Rights" to keep. **Participation in this study is voluntary.** You may refuse to answer any question or discontinue your involvement at any time without penalty or loss of benefits to which you might otherwise be entitled. Your decision will not affect your future relationship with UCI or your quality of care at the UCI Medical Center.

If, during the course of this study, significant new information becomes available that may relate to your willingness to continue to participate, this information will be provided to you by the research team listed at the top of the form.

Your signature below indicates you have read the information in this consent form and have had a chance to ask any questions you have about this study.

Note: As the research described in this form involves your protected health information (PHI), you will be asked to sign separate UC HIPAA Research Authorization form for the use of your PHI.

I agree to participate in the study.

_____ _____
Subject Signature **Date**

_____
Printed Name of Subject

_____ _____
Signature of Person Obtaining Informed Consent **Date**
(Individual must be listed on Page 1 of this consent)

_____
Printed Name of Person Obtaining Informed Consent

UNIVERSITY OF CALIFORNIA, IRVINE
Experimental Subject's Bill of Rights

The rights listed below are the right of every individual asked to participate in a research study. You have the right:

1. To be told about the nature and purpose of the study.

2. To be told about the procedures to be followed in the research study, and whether any of the drugs, devices, or procedures is different from what would be used in standard practice.

3. To receive a description of any side effects, discomforts, or risks that you can reasonably expect to occur during the study.

4. To be told of any benefits that you may reasonably expect from the participation in the study, if applicable.

5. To receive a description of any alternative procedures, drugs, or devices that might be helpful, and their risks and benefits compared to the proposed procedures, drugs or devices.

6. To be told of what sort of medical treatment, if any, will be available if any complications should arise.

7. To be given a chance to ask any questions concerning the research study both before agreeing to participate and at any time during the course of the study.

8. To refuse to participate in the research study. Participation is voluntary. You may refuse to answer any question or discontinue your involvement at any time without penalty or loss of benefits to which you might otherwise be entitled. Your decision will not affect your right to receive the care you would receive if you were not in the experiment.

9. To receive a copy of the signed and dated written consent form and a copy of this form.

10. To be given the opportunity to freely decide whether or not to consent to the research study without any force, coercion, or undue influence.

If you have any concerns or questions regarding the research study you should contact the research team listed at the top of the consent form.

If you are unable to reach a member of the research team and have general questions, or you have concerns or complaints about the research study, research team, or questions about your rights as a research subject, please contact the UCI's Human Research Protections unit in the Office of Research by calling (949) 824-6068 or (949) 824-2125 Monday – Friday, 8 am – 5 pm; or by e-mail at IRB@research.uci.edu; or by writing us at 141 Innovation Drive, Suite 250, Irvine, CA 92697.