PHASE II INFORMATION LETTER FOR CAREGIVERS OF INDIVIDUALS WITH LAL-D

Study Title
The Lysosomal Acid Lipase Deficiency-Health Index (LALD-HI): Development of a Caregiver-Reported Outcome Measure for Lysosomal Acid Lipase Deficiency (LAL-D)

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This information sheet describes a research study that is being conducted by Dr. Chad Heatwole from the University of Rochester's Department of Neurology. The purpose of this study is to determine the symptoms and issues that are most important to individuals with lysosomal acid lipase deficiency (LAL-D) as reported by a caregiver, in order to develop and validate a disease-specific caregiver-reported outcome measure for use in clinical trials.

This study is being conducted in multiple phases. This information letter outlines participation in Phase II where you will have the opportunity to provide your insights about the individual with LAL-D for whom you currently provide care or have cared for in the past about their experiences living with LAL-D through an online linked survey. We anticipate that 150 subjects will participate in this study. This survey will take approximately 20-30 minutes to complete, and all responses will be strictly confidential. Your survey responses will be completely anonymous. The inclusion and exclusion criteria for this phase of our study is as follows:

Inclusion Criteria
You must 1) currently care for or have cared for an individual with a diagnosis of LAL-D; 2) be 18 years or older; 3) read and understand English; and 4) reside in the United States, Canada, United Kingdom, European Union, or Australia.

Exclusion Criteria
You will not be selected to participate in this study if you 1) do not meet all the inclusion criteria above, 2) do not have access to internet, or 3) are unable to provide informed consent.

The survey contains some personal questions about you and the individual with LAL-D for whom you currently care for or have cared for in the past, in addition to the specific symptoms that have the greatest impact on their life. The symptoms included in this survey were previously identified as important through interviews with caregivers of individuals with LAL-D. If possible, we would like you to complete the entire survey, but you may skip any questions that you do not feel comfortable answering.

Some of the survey questions may be upsetting or make you feel uncomfortable. You can skip any of the questions you do not want to answer. Because this study involves collecting the health information of the individual with LAL-D, there is a potential for invasion of privacy or breach in confidentiality. To minimize this risk, your survey responses will be anonymous. All of the information that we collect will be stored in a secure manner, and only study team members will have access to it. There are no other expected risks.

You will not receive direct benefits from participating in this study. The data from these surveys may assist in the development of a LAL-D specific, caregiver-reported outcome measure to be used in clinical trials and patient care. This survey will ultimately allow future LAL-D therapies to be directly evaluated based on symptoms that you (and others) identify.

Confidentiality of Data: The University of Rochester makes every effort to keep the information collected from you private. In order to do so, your survey responses will be anonymous. Survey data will be stored indefinitely. All study data will be stored in a secure manner and accessible only to the study team. Sometimes, however, researchers need to share information with people that work for the University, study sponsors, or regulators. If this does happen, we will take precautions to protect the information you have provided. Results of the research may be presented at meetings or in publications, but your name will not be used.

The University of Rochester is receiving funding to conduct this research study. The University of Rochester and Chad Heatwole, principal investigator, may or may not receive licensing fees through the eventual development of the LALD-HI. You will not be paid for participating in this study. There will be no cost to you for participating in this study.

Authorization to Use Health Information: In order to collect study information, we have to get your permission to use and store your health information. Your name and other personally-identifiable information will NOT be collected in the survey data. Your permission to use anonymous health information for this study will not expire. We will keep the anonymous information we collect indefinitely.

Your participation in the study is completely voluntary. You are free not to take part or to withdraw at any time, for whatever reason. No matter what decision you make, there will be no penalty or loss of benefit to which you are otherwise entitled. In the event that you do withdraw from this study, the information you have already provided will be kept in a confidential manner.

For more information concerning this research or if you feel that your participation has resulted in any emotional or physical discomfort, please contact the study coordinator:

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Please contact the University of Rochester Research Subjects Review Board at 265 Crittenden Blvd., CU 420628, Rochester, NY 14642, Telephone: +1 585 276-0005 or +1-877 449-4441 for the following reasons:

- You wish to talk to someone other than the research staff about your rights as a research subject;
- To voice concerns about the research;
- To provide input concerning the research process;
- In the event the study staff could not be reached.

Thank you for your interest and your time!