INFORMATION SHEET FOR CAREGIVERS OF INDIVIDUALS WITH NEURODEVELOPMENTAL DISORDERS

Study Title:
Development of a Caregiver-Reported Outcome Measure for Neurodevelopmental Disorders

Principal Investigator:
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This information sheet describes a research study that is being conducted by Dr. Chad Heatwole, MD, MS-CI, from the University of Rochester Department of Neurology in Rochester, New York, United States.

The purpose of this study is to develop and validate a disease-specific, caregiver-reported outcome measure for clinical trials of individuals with the following Neurodevelopmental Disorders (NDDs): Smith-Magenis Syndrome (SMS) / RAI1, SCN2A, SHANK3 / Phelan McDermid Syndrome (PMS), SYNGAP1 deficiencies and Fragile X Syndrome (FXS). The study is being conducted in multiple phases. We are contacting you regarding Phase 2, which involves a survey regarding potential symptoms of importance to patients with NDDs, as reported by caregivers. We are seeking caregivers, ages 18 and up, of individuals with one of the above NDDs to take part in Phase 2.

As part of Phase 2, if you agree to participate, you will have the opportunity to provide your insights about NDDs through the linked survey. The information from this study will be used to help develop a NDD-specific caregiver-reported outcome instrument for individuals with NDDs, to be used in clinical trials and patient monitoring. This instrument will, ultimately, allow future therapies in NDDs to be directly evaluated based on symptoms and domains that you (and others) identify as important.

If you are willing to participate in this research, you may do so by going online to **https://redcap.link/NDDCaregiverSurvey** and completing the survey. The survey will take approximately 20 minutes to complete and all responses will be completely anonymous. The survey contains some personal questions about you, your household, and the specific issues and symptoms from NDDs that have the greatest impact on the life of the individual for whom you provide care. The issues and symptoms included on this survey were previously identified as being important by caregivers of individuals with NDDs. If possible, we would like you to complete the entire survey, but you may skip any questions that you don't feel comfortable answering.

If you prefer, the survey can also be completed on paper or over the phone. If you prefer either of these methods, please contact one of the study coordinators (contact information provided at the end of this sheet).

There is a minimal risk associated with this research. Some of the survey questions may be upsetting or make you feel uncomfortable. You can skip any questions you do not want to answer. Because this study involves collecting health information of the person under your care, there is a potential for invasion of privacy or breach in confidentiality. To minimize this risk, the survey will not collect or store any identifying information about you or the person under your care. Your survey responses will be anonymous and all of the information we collect will be stored in a secure, locked location. There are no other expected risks. There are also no expected benefits. Results of this anonymous research may be presented at meetings or in publications. We will keep the anonymous information we collect about you and the person you care for indefinitely.

The University of Rochester is receiving funding for conducting this research. You will not be paid for participating in this study. There are no expected costs to participating in this study.

Participation in this study is voluntary: You are free not to participate or withdraw at any time, for whatever reason without penalty or loss of benefit to which you are entitled. In the event that you do withdraw from this study, the information you have already provided will be kept in a secure manner.

For more information or concerns regarding this research, please contact:
Jennifer Weinstein at jennifer.weinstein@chet.rochester.edu
OR
Charlotte Engebrecht at charlotte.engebrecht@chet.rochester.edu.

Please contact the University of Rochester Research Subjects Review Board at 265 Crittenden Blvd, CU 420628, Suite 1-250, Rochester, NY 14642, Telephone (585) 276-0005 or (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!