CLINICALLY MEANINGFUL PATIENT-REPORTED OUTCOMES IN PKU:

ONLINE SURVEY

CONSENT FORM

Who is conducting the study?

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Throughout this document

"You" refers to "you", "your child" or "your ward"; "we" means the physicians and other staff conducting the study. If you have been invited to take part in this survey as a parent of a child with PKU, "you" refers to your child in this document.

Invitation

You are being invited to take part in this research study because you have been diagnosed with Phenylketonuria (PKU).

What is the purpose of this study?

We would like to learn more about your experience and your perspective on treatment with Kuvan. We would like to better understand which outcomes are most important to you when it comes to Kuvan. We believe that the results of this research will inform decision-makers for better treatment and care.

What information will be collected?

- What country are you from
- Your age, gender and severity of your PKU
- How do you manage your PKU
- Your experience/opinion regarding Kuvan treatment

There are 28 questions, some of them have answer choices and some are open-ended (you type your answer). The survey should take approximately 15-20 minutes to complete.

Is there any way being in this study could be bad for you?

We believe that here is nothing that could harm you or be bad for you in this study. There are no physical risks or discomforts because the study only involves entering your information online. There is no testing involved. We will not ask you to provide any information that can directly identify you. The small risk of release of personally identifiable information due to the rarity of the disease is very unlikely but possible.

There will be no names or other personal identifiers in the survey database. When the survey is completed, we will export the information into Excel format for the statistical analysis. This information will be saved on the hard drive in Dr.Stockler's computer at Children's Hospital, British Columbia, Canada; University of British Columbia. This computer is part of Provincial Health Authority (PHSA) network with limited, password-protected access.

What are the benefits of participating?

We hope that information learnt in this survey could be used to improve health care decision making procedures in PKU management.

How the results of the study will be shared?
We plan to collect the survey data for about 6 months – 1 year, depending on how many answers we receive. After the study is finished, we will close the survey and will analyze the data. We hope to present the results of this survey at one of the CanPKU days and also at Garrod meeting. We also plan to publish the results in medical literature.

How will your privacy be maintained?
Your confidentiality will be respected. We will use REDCap as a secure online platform to collect information. Your information will be entered into a secure, limited access, digital database called REDCap and hosted on secure server at Canadian Family Research Institute (CFRI).
Every participant will be given study ID. We will not collect any personal identifiers. You will not be identified in any reports of the completed study. The information will be used exclusively for the research purposes.

Will you be paid for your time/ taking part in this research study?
We will not pay you for the time you take to be in this study.

Who can you contact if you have questions about the study?
If you have any questions or concerns about what we are asking of you, please contact the study leader or the study coordinator. The names and telephone numbers are listed at the top of the first page of this form.

Who can you contact if you have complaints or concerns about the study?
If you have any concerns or complaints about your rights as a research participant and/or your experiences while participating in this study, contact the Research Participant Complaint Line in the UBC Office of Research Ethics at 604-822-8598 or if long distance e-mail RSIL@ors.ubc.ca or call toll free 1-877-822-8598.
Taking part in this study is entirely up to you. You have the right to refuse to participate in this study. If you decide to take part, you may choose to pull out of the study at any time without giving a reason and without any negative impact on you.

IF THE QUESTIONNAIRE IS COMPLETED, IT WILL BE ASSUMED THAT CONSENT HAS BEEN GIVEN