

University of South Carolina

Consent Information

Study Title: Family Experiences and Treatment Priorities in Pediatric Urea Cycle Disorders

Key Information

You are invited to take part in a minimal-risk research study at the University of South Carolina. The study is led by Dr. Greg Trevors in the Department of Educational and Developmental Sciences. NUCDF may help with recruitment and community engagement.

The purpose is to better understand caregiver experiences, treatment burden, ongoing risk, treatment decision-making, and treatment preferences among families of children with a urea cycle disorder (UCD), such as OTC deficiency, CPS1 deficiency, citrullinemia, argininosuccinic aciduria, or arginase deficiency. Participation is voluntary and will not affect your medical care, services, NUCDF involvement, or access to treatments.

Why You Are Being Asked

You are being asked because you are an adult parent, guardian, or caregiver of a child under 12 years old with a urea cycle disorder. Bereaved caregivers and families whose child received a liver transplant are also welcome and answer about the period when the child was under 12.

What You May Do

- Complete an online survey, about 15-20 minutes, with optional extra questions taking about 10 minutes.
- Optionally join a one-on-one interview, about 30-60 minutes.
- Optionally join a virtual group listening session, about 90 minutes.
- Optionally send a written response or voice memo, about 15-30 minutes depending on what you choose to share.
- Optionally answer brief follow-up questions if clarification is needed.

You can choose which parts to do. You may skip any question and stop at any time.

Recording and Transcription

With your permission, interviews, listening sessions, and voice memos may be audio recorded and transcribed. You may decline recording. Transcripts will be reviewed, coded, and summarized to identify common themes. Names and direct identifiers will be removed when possible.

Risks or Discomforts

Risks are expected to be minimal. Some questions may bring up stress, grief, frustration, or worry when discussing illness, hospitalization, treatment burden, family strain, finances, decision-making, or future therapies. You may skip questions, take a break, or stop.

There is also a risk of loss of confidentiality. Because urea cycle disorders are rare, some of them very rare, and family experiences can be specific, complete anonymity cannot be guaranteed. In group listening sessions, other participants will hear what you share. The study team will ask everyone to respect privacy, but cannot guarantee what other participants will do.

Benefits

You may not directly benefit. The study may help researchers, clinicians, advocacy organizations, and treatment-development audiences better understand family experiences and priorities.

Costs and Payment

There is no cost to participate other than your time. Participants may receive gift-card compensation for completed or substantially completed activities: survey \$30; interview \$50; listening session \$100; optional voice memo/written narrative \$20. You do not need to complete all activities to receive payment for the parts you complete. You may decline compensation. Payment information, such as name, email, or mailing address, may be collected and stored separately from research responses when possible.

Privacy and Confidentiality

Research records may include survey responses, notes, recordings, voice memos, transcripts, coded data, and contact/payment information. Records will be stored using secure, university-approved systems and accessed only by authorized study personnel when possible.

Information from this study may be published or presented, but names and direct identifiers will not be used unless you give separate explicit permission. The University of South Carolina IRB and study investigators may access study records as allowed by law and university policy. If you take part in more than one activity and give permission on the separate contact form, we may connect what you share in an interview or listening session with your survey answers using a study ID only; your name is never attached to the survey data.

Use of Information

Study findings may be used for research reports, academic publications, conference presentations, educational materials, advocacy or community summaries, and summaries for clinicians, researchers, treatment developers, or regulatory audiences. Findings will be reported in aggregate. De-identified information may be retained for future analysis or publication. De-identified survey data and coded summaries (not recordings or full transcripts) may be shared with other qualified researchers, including advocacy organizations, academic institutions, or organizations developing treatments, or placed in a research data repository, after identifying details are removed.

Voluntary Participation and Withdrawal

Taking part is voluntary. You may decline, skip any question, or stop at any time without penalty. If you withdraw, the study team will stop collecting new information from you. Information already collected may still be used in de-identified form unless you request removal and it is still practical to remove it.

Questions

Study questions: Dr. Greg Trevors, Department of Educational and Developmental Sciences, University of South Carolina. Email: trevorsg@mailbox.sc.edu. Phone: (803) 414-8877.

Questions about your rights as a research participant: Lisa Johnson, Director, Office of Research Compliance, University of South Carolina, 1600 Hampton Street, Suite 805, Columbia, SC 29208. Phone: (803) 777-6670. Email: LisaJ@mailbox.sc.edu.

Consent

By continuing to the survey, interview, listening session, or audio/written response, you confirm that you have read this information, had a chance to ask questions, and agree to participate. For recorded activities, you will also be asked whether you agree to audio recording and transcription.