



Volunteers needed to rate an online questionnaire on health-related quality of life

As part of the study "*Development of a patient-reported outcome to measure the health-related quality of life of children, adolescents, and young adults with cystinosis: QUALIFY-US*" of the Department of Medical Psychology at the University Medical Center Hamburg-Eppendorf (UKE), we invite children, adolescents, and young adults with cystinosis as well as their parents to participate in an online questionnaire on health-related quality of life (HrQoL) in young people with cystinosis.

Why is the study being conducted?

The effects of the disease and the strict treatment plan for cystinosis can have a negative impact on HrQoL. There is no disease-specific questionnaire to assess HrQoL in young people with cystinosis. The aim of the study is to develop and validate a disease-specific questionnaire to assess HrQoL. A validated, disease-specific questionnaire is an important tool for improving patient-centered care or evaluating a new drug or therapy.

What has happened up to this point?

We have interviewed young individuals affected by cystinosis and their parents to explore their experiences with the condition and its effects on their HrQoL. Drawing insights from these interviews, we have developed an initial set of questions for an online survey. We welcome your thoughts on the relevance of these questions to your life with cystinosis and whether there are any additional aspects you would like to include or if any questions need to be rephrased.

Who can participate in the study?

We are looking for :

- ✚ **Children, adolescents, and young adults** with a confirmed diagnosis of cystinosis aged **8-26 years** as well as at least one of **their parents or a legal guardian living in the US.**
- ✚ **Parents or legal guardians of 0-7 years old children** with a confirmed diagnosis of cystinosis **living in the US.**

What does my participation include?

You will receive a link to an online survey by e-mail. The survey will take approximately 45 minutes to complete. As compensation, each participating family will receive a voucher worth \$17.25.

If you would like to participate or have any questions about the study, please get in touch with **Mona Suck** (e-mail: quality-of-life@uke.de).

Your participation is crucial in providing a deeper understanding of the impact of cystinosis on daily life and overall well-being. By sharing your experiences, you contribute directly to developing a questionnaire that genuinely reflects the concerns and challenges patients like yourself face.

We appreciate your support!