



**The Pediatric Neurology Research Group at the University of California, San Diego (UCSD) is conducting the following research study on cystinosis:**

**Outcome and Quality of Life in Adults with Cystinosis**

**About the Study:** Angela Ballantyne, Ph.D. and Doris Trauner, M.D., of the UCSD School of Medicine, are conducting a questionnaire-based research study to learn more about outcome and health-related quality of life in adults with the genetic-metabolic disorder, cystinosis. The purpose of this study is to gather vital information on outcome and quality of life in the ever-expanding population of adults living with cystinosis. The information obtained from this study can also be used to weigh the risks versus benefits of new therapies or treatments for cystinosis.

**What the Study Involves:** This is an online, questionnaire-based study that examines quality of life and outcome in adults with cystinosis. Participants will be asked to log in to a secure, online assessment center and complete a series of questionnaires that will take approximately 1 to 2½ hours. Questions will be asked regarding demographic background, health, medical issues, quality of life, and outcome in cystinosis. Participation in the study is strictly confidential. Data from all participants will be combined, and you will not be identified by name in any publication or teaching activities.

**Who may participate?** Two groups of participants are being recruited to take part in this online study. You are eligible to participate if:

**(a)** you are an adult over 18 years of age living with cystinosis,

**OR**

**(b)** you are a parent or nearest living relative of an adult who had cystinosis and passed away within the past 10 years.

Participation in this research study is completely voluntary. If you would like to participate in the Cystinosis Adult Outcome study or if you would like more information, please contact 858-822-6800, or email Jennifer Crowhurst at [jcrowhurst@ucsd.edu](mailto:jcrowhurst@ucsd.edu).