



MASSACHUSETTS
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MEDICAL SCHOOL

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Florian Eichler, MD
Associate Professor of Neurology
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Hello,

I am writing to tell you about a multi-part research study I am conducting at Massachusetts General Hospital, titled ***Clinical Trial Readiness for Distal Myopathy in Nephropathic Cystinosis***. The purpose of this portion of the study is to create a **disease-specific assessment tool** for the cystinosis patient community called Distal Myopathy Function Score (DMFS). Your input is valuable and will eventually validate this cystinosis tool. Developing the DMFS is a step-wise process, and we may send an updated DMFS survey as we continue to refine our tool in the near future.

I am informing you about the **opportunity to participate** in this study because you are engaged in the cystinosis community. The Cystinosis Research Foundation is helping us recruit patients and distribute information about the study through social media and other platforms.

If you choose to participate in the study, please **email** the research team at rtduong@partners.org. We ask that you provide *your name, date of birth, and email address*. This information will be kept on a password-protected computer in a locked office at Massachusetts General Hospital for participant contact documentation. By doing this, we will do our best to protect your confidential information. Still, there is always a small risk of an unintended breach of confidentiality.

You will receive an emailed link to the survey. The **DMFS survey** should take you **about 3-5 minutes** to complete. We estimate that we will distribute this survey to 50-100 patients. Your answers are anonymously recorded in our system. The survey that we are sending you is specific to your email address and allows us to verify whether you have completed the survey. We may ask that you complete the survey additional times as we revise the DMFS based on periodic data analyses.

You will not receive any personal health benefits as a result of your participation in this research study. We hope that the results will benefit the treatment and care of patients with cystinosis in the future. Your participation is **completely voluntary**. Whether you participate or not will have no effect on the medical care you receive here at

Massachusetts General Hospital or Partners Healthcare, either now or in the future, and will not impact any benefits you receive now or have a right to receive. Your survey will not become a part of your medical record, and your completed anonymous survey will only be accessible by the study staff.

Please contact the study coordinator Rachel Duong at (617) 724-1330 if you would like to learn more about the study.

If you'd like to speak to someone not involved in this research about your rights as a research subject, or any concerns or complaints you may have about the research, contact the Partners Human Research Committee at (857) 282-1900.

We are required by the Health Insurance Portability and Accountability Act (HIPAA) to protect the privacy of health information obtained for research. This is an abbreviated notice, and does not describe all details of this requirement. During this study, identifiable information about you or your health will be collected and shared with the researchers conducting the research. In general, under federal law, identifiable health information is private. However, there are exceptions to this rule. In some cases, others may see your identifiable health information for purposes of research oversight, quality control, public health and safety, or law enforcement. We share your health information only when we must, and we ask anyone who receives it from us to protect your privacy.

Please contact my research coordinator Rachel Duong at (617) 724-1330 with any questions about this research study.

Sincerely,

A handwritten signature in black ink, appearing to read 'Florian Eichler', written in a cursive style.

Florian Eichler, MD