

CLINICAL RESEARCH STUDY FOR MUCOLIPIDOSIS TYPE IV

WHY IS THE ML4 FOUNDATION COLLABORATING WITH MGH ON THIS STUDY?

We plan for this study to assist in the design of clinical research trials to help test potential treatments for Mucopolysaccharidosis Type IV. Clinical research trials are trials of treatments in people with the disease. Before clinical trials are approved by the FDA, we must present a highly developed picture of the disease progression at all stages and ages to the FDA, indicating to them that we understand Mucopolysaccharidosis Type IV fully. This demonstrates that we understand how and when a treatment will be effective. For this reason, your participation in research studies like this are crucial to the development of effective treatments.

JOIN US TO HELP PHYSICIANS GATHER CLINICAL PATIENT DATA FOR MUCOLIPIDOSIS TYPE IV. THIS DATA IS ESSENTIAL IN ORDER TO BUILD MEANINGFUL TREATMENTS!

WHO IS ELIGIBLE FOR THIS STUDY?

Children & adults of between 2 and 99 years of age with a confirmed diagnosis of Mucopolysaccharidosis Type IV

WHAT WILL BE REQUIRED IF YOU DECIDE TO PARTICIPATE?

- Read and sign the consent form
- Provide past medical records to the Study Team
- Complete a questionnaire about you or your child's development and symptoms related to Mucopolysaccharidosis Type IV
- If you are able, you may also be asked to participate in annual neurological and ophthalmological examination by a physician investigator during a visit to Massachusetts General Hospital and Boston Children's Hospital.

Our Study Team sees patients at Massachusetts General Hospital in Boston and is creating a disease-specific severity scale for Mucopolysaccharidosis Type IV. If you are interested in participating, please bring any copies of medical records, examination results, CDs of MRIs and other imaging with you to the site visits.

HOW DO I GET STARTED?

You can contact the Study Team by phone or e-mail for details about the research:
Massachusetts General Hospital – Center for Rare Neurological Diseases Research

Research Coordinator: Madeline Debono 617-643- 4218 or mdebono@mgh.harvard.edu

Principal Investigator: Albert Misko, MD, PhD



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