

CLINICAL RESEARCH STUDY FOR MUCOLIPIDOSIS TYPE IV

We plan for this study to assist in the design of clinical research trials to help test potential treatments for Mucopolidosis Type IV.

Join us to help physicians gather clinical patient data for Mucopolidosis Type IV.

Who is eligible for this study?

- Children & adults of between 2 and 99 years of age with a confirmed diagnosis of Mucopolidosis 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 Mucopolidosis 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 aiming to create a disease-specific severity scale for Mucopolidosis 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.

You can contact the Study Team by phone or e-mail for details about the research:

Massachusetts General Hospital – Center for Rare Neurological Diseases

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Principal Investigator: Albert Misko, MD, PhD



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