



GNE Myopathy Clinical Studies at NIH

A Natural History Study of Patients with GNE Myopathy

Frequently Asked Questions about the Study

Where will I have to go to participate in the study?

You will be admitted to the [NIH Clinical Center](#) for this study.

Will I get paid to participate in this study?

You will not receive any direct compensation for your participation.

What are the benefits to participating in this study?

By participating in this study, you will help to improve our understanding of GNE myopathy, which is an important contribution to families and individuals affected by this condition. This study will provide information that will help all clinical trials that explore new therapies for GNE myopathy.

Will travel to the NIH Clinical Center be covered?

Yes, for participants within the United States and Canada. For participants whose home of record is outside the United States and Canada, travel expenses from a U.S. port of entry may be covered. In some instances, NIH will pay for expenses that involve travel from the home of record to the NIH site. NIH will not pay for expenses that involve alternate routes, unnecessary stops or delays.

Does participating in the GNE myopathy Natural History Study mean that I am automatically enrolled in the ManNAc trials?

No it does not. The eligibility criteria for the ManNAc trial might be different from those for this natural history study.

Why should I stop taking any medications while I participate in this study?

Yes, you need to stop taking ManNAc, sialic acid, IVIG and siallyllactose.

I have multiple family members with GNE myopathy, can we all participate?

Yes! As long as each person has evidence of a diagnosis of GNE myopathy and signs informed consent, you may all participate. Furthermore, we can coordinate to bring you and your siblings or friends to the study at the same time.

How do we participate?

You simply need to email or call us if you are interested in participating:

To determine your eligibility, send your medical records, including neurology note and genetic testing results confirming the diagnosis of GNE myopathy to:

Kennan Bradley

Email: kennan.bradley@nih.gov

Phone: (301) 827-7746

Fax: (301) 402-0006

How do I obtain genetic testing?

Genetic testing is ordered by your physician when he or she suspects that you have GNE myopathy. You can contact us if you have questions. Here is information on how to obtain genetic testing; [Genetic Testing Registry](#)

Where will the study take place?

At the National Institutes of Health Clinical Center in Bethesda, Maryland, USA. For more information, see: <https://clinicalcenter.nih.gov/about1.html>

Where can I get more information about GNE myopathy?

[Inclusion body myopathy 2](#) [ghr.nlm.nih.gov]

[GNE Myopathy](#) [ommbid.mhmedical.com]

[Advancement of Research for Myopathies \(ARM\)](#) [hibm.org]

[Neuromuscular Disease Foundation \(NDF\)](#) [ndf-hibm.org]

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