Clinical studies:

Are Confidential. Participation in a clinical study is confidential. Participants' names and other identifying information will not be shared in reporting data.

Involve No Costs. Participants will be reimbursed for reasonable out-of-pocket travel costs, including travel to and from the study site, 1-2 night hotel stay per visit and meals for the participant and one companion. Participants will also receive medical care related to the study at no cost.

Are Important to the Community. Participation in clinical studies contributes to research and can benefit others affected by GNE myopathy. However, there may be no clear benefit for patients who participate.

May Involve Risks. Any clinical research study can involve risks, such as the risk of side effects from the study treatment. Please discuss the specific risks associated with this study with your study doctor.

For more information, visit
www.clinicaltrials.gov
(search using NCT02377921)

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Talk to Your Doctor About Genetic Testing for GNE Myopathy:

Physicians can order a genetic test for GNE myopathy at no cost for people with suspected symptoms. Physicians and office staff can contact **GNEM@engagehealth.com** for more information.

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GNE Myopathy Research Updates for Patients

Learn about a Phase 3 clinical research study in GNE myopathy, also known as hereditary inclusion body myopathy (HIBM)

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Phase 3 Clinical Research Study

A Phase 3 clinical study in people with GNE myopathy, also known as hereditary inclusion body myopathy (HIBM) or Nonaka myopathy is underway. The study will be open to people aged 18-55 who can walk greater than or equal to 200 meters (about 200 yards, the length of 2 football fields) in six minutes without using a cane, crutches, walker, or other assistive devices. Ankle foot orthoses are allowed.

Additional criteria are required for participation, and not all people will meet the requirements for this study. Patients who take sialic acid, ManNac, St. John's Wort (or anything else that creates sialic acid in the body) will be asked to discontinue their medications for 60 days before the screening visit.

Talk to your doctor for more information about this Phase 3 global study

What should I know about the Phase 3 aceneuramic acid ER study

- The Phase 3 study will collect information on the safety and efficacy of aceneuramic acid (sialic acid) extended release (ER) in people with GNE myopathy.
- Aceneuramic acid ER is an investigational drug.
- After your initial visit to the study site, taking part in the study requires a one- to two-day visit to the study site every two months during the 48-week study duration, for a total of eight visits.
- The study will be placebo-controlled, which means half of enrolled patients will receive aceneuramic acid ER and half will receive a placebo (sugar pill).
- After the study, all participants may have the opportunity to take part in an extension study in which they will receive aceneuramic acid ER.
- Reasonable travel expenses to clinical study sites within acceptable distances will be covered.

For more information, visit
www.clinicaltrials.gov
(search using NCT02377921)