NIMBLE STUDY

NIMBLE Study Enrolling: Join Now!

Do you have generalized Myasthenia Gravis or know someone with Myasthenia Gravis?

Talk to your doctor now about NIMBLE clinical study

Regeneron Protocol Number: R3918-MG-2018

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What is NIMBLE?

The NIMBLE study is exploring if a combination of two investigational study drugs given as a once monthly subcutaneous injection for adults with generalized Myasthenia Gravis is effective and well-tolerated.

If you are diagnosed with generalized myasthenia gravis (gMG), please know that you are not alone in your medical journey. gMG is a rare autoimmune disorder caused by the autoantibodies produced by the immune system that attack the body's own cells. Antibodies are proteins naturally found in your blood that help fight infections. These antibodies activate a pathway in the immune system called the complement pathway which leads to disruption of the communication between nerves and muscles, so that the muscle cannot function normally. gMG affects between 50 and 200 people per million worldwide.

There are treatments, but presently no known cure.

You may have heard from your doctor about clinical studies that are taking place to find potential treatment options for gMG. As always, your primary doctor is the best person to discuss this with, but we hope the information in this brochure may help to answer some of your questions about joining clinical studies.

See if the NIMBLE study may be right for you!

What is a clinical study?

A clinical trial or study is the way we evaluate and compare different study drugs to help patients with various diseases. The study drugs are called "investigational" as they are not approved in this country. The investigational study drugs only enter clinical trials after researchers test them in the laboratory. Clinical studies show if they are well-tolerated and effective and provide information on any side effects. All clinical research studies must be reviewed and approved by a regulatory agency to ensure the health, privacy, and safety of study participants.

Am I eligible to participate?

You may be eligible to take part if you have been diagnosed with gMG and are:

- 18 years of age or older (or of legal adult age in your country)
- Currently experiencing signs and symptoms despite standard of care treatment
- Positive for anti-AChR or anti-LRP4 antibodies (please ask your doctor or the NIMBLE study team if you are unsure)
- Receiving an acetylcholinesterase inhibitor and an immunosuppressant drug for gMG (some exceptions may apply)

There are other requirements to participate, including the need for certain vaccines such as a meningococcal vaccine. Such vaccinations are known to reduce risks of certain infections while receiving C5 blocking agents.

If you have questions or concerns over vaccinations, please contact your doctor for more information.

You may not be eligible if you:

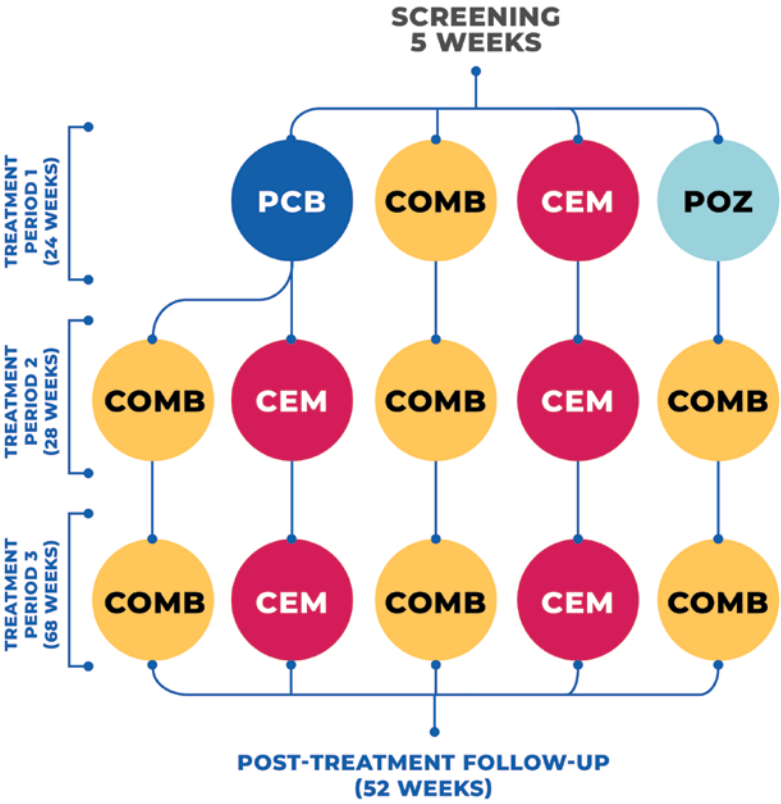
- Have an antibody profile positive only for MuSK and negative for anti-AChR and anti-LRP4 (if you are unsure, please ask your doctor)
- Have had a thymectomy within 12 months or are planning to have a thymectomy
- Have certain medical conditions, such as malignant thymoma, cancer, or HIV infection
- Are pregnant, planning to become pregnant, or breastfeeding

There may be other reasons why this study may not be suitable for you – your doctor or the NIMBLE healthcare team can help you understand why.

What can I expect during the study?

Taking part in a clinical study is a commitment. The study team will share information on what to expect, as well as your roles and responsibilities if you join the NIMBLE study.

If you would like to take part in this study, you will visit the clinic on a regular basis to receive the study drugs and undergo other health- and study-related procedures and assessments. This study will last approximately 3.5 years:



PCB: Placebo | COMB: Combination | CEM: Cemdisiran | POZ: Pozelimab

Participants may also be compensated for travel and accommodation costs.

The length of your participation depends on how you respond to the study drugs and is decided by you and your study doctor. Your health and response to the study drugs will be closely monitored throughout the study.

Always remember, taking part in a clinical study is 100% voluntary (your choice). Education and additional services will be provided to you throughout the duration of the study. You may choose to leave the study at any point without it affecting your normal health care.

What are the potential study drugs I will be taking?

If you meet all the requirements to participate in this study, there is approximately 70% chance of receiving active study drugs and approximately 30% chance of receiving placebo in the first treatment period. After this, all participants will receive active study drugs. You may receive the following study drugs:

- Investigational drug 1 (pozelimab): A monoclonal antibody that targets your complement component 5 (C5). In people with gMG, the action of C5 damages the neuromuscular junction. Monoclonal antibodies are laboratory-made proteins that are meant to act as the natural ones found in your immune system.
- Investigational drug 2 (cemdisiran): An RNAi therapeutic that suppresses the production of complement proteins (specifically C5).

- Combination therapy: Combines both investigational study drugs for complete suppression of the complement C5 system. This is being studied to see if this may reduce the damage caused by C5 at the neuromuscular junction, reduce symptoms, as well as lead to lower and less frequent dosing. In a study of healthy volunteers, the combination therapy was generally well tolerated.
- Placebo: A placebo looks like the investigational study drugs being studied but has no active ingredients.

The investigational study drugs are called “investigational” as they have not been approved to treat gMG. If you are on other treatments to manage symptoms of gMG, you may be able to continue taking your regular treatment, depending on the study requirements. The study doctor will discuss this with you.

What are the benefits and unintended side effects?

By participating in this study, you will contribute towards improving our medical knowledge of myasthenia gravis and in the future, this may benefit others diagnosed with myasthenia gravis.

If you choose to participate, you may benefit from additional check-ups on top of your regular care and will receive the study drugs at no cost to you.

As with all study drugs, there are possible side effects when taking them. You will be provided with an Informed Consent Form that explains any possible unintended side effects. It is also possible that the study drugs may affect you in unknown ways.

How can I participate?

Thank you for your interest in NIMBLE. To learn more, please contact the study team.

The study team is always available and happy to discuss this study with you. You can contact them with the details provided below.