myasthenia gravis association
MGA
of the heartland

SPECIAL EDITION

MYASTHENIA GRAVIS ASSOCIATION NEWSLETTER

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Clinical Trials 101

Clinical Trials

Clinical trials play a major role in advancing treatment options for people living with myasthenia gravis (MG). Many of the therapies available today became possible because patients chose to participate in research studies. At the same time, clinical trials can feel overwhelming or intimidating, especially when you are already managing a chronic condition. Questions about safety, eligibility, placebo groups, travel, and daily life are extremely common.

We are excited to share with you our special edition clinical trials newsletter! Some pieces of this article can be found in our recent blog post on the MG Digest. Don't forget to check out the blog each Friday for new posts.

This guide is designed to answer some of the most frequently asked questions about MG clinical trials and help individuals feel more informed when discussing research opportunities with their healthcare team.

What Is a Clinical Trial?

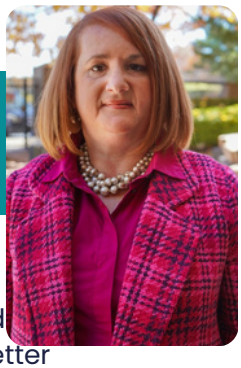
A clinical trial is a research study involving human participants that evaluates the safety and effectiveness of a treatment, medication, medical device, or intervention. In MG, clinical trials often focus on:

- New medications
- New uses for existing medications
- Symptom management
- Biomarkers and disease monitoring
- Treatment combinations
- Quality of life improvements

Clinical trials are carefully regulated and follow strict safety guidelines overseen by organizations such as the FDA and institutional review boards (IRBs).

A MESSAGE FROM ALLISON

Hope for the Future



As we were planning content and programs for 2026, one of our volunteers with experience in the pharmaceutical industry shared the idea to create a Clinical Trials newsletter. We thought what better time to do this than in May which is Clinical Trials month.

Also, no better time to do this than the present when there are a multitude of clinical trials in the myasthenia gravis (MG) space and educating our community on them could become a fulltime job. I recognize there are several things we thing need to consider when it comes to clinical trials such as; the distance to the site, time restraints, process of dosing, for some childcare for appointments and travel, severity of one's disease state, and subtype of MG.

However on the flip side of these considerations is the fact that individuals living with MG are needed to participate in these trials so we can advance medicine, uncover additional research and potential treatments and bring us closer to a cure. Because there are so many current trials for MG, one might say the space has become crowded however I see that as excitement and movement and steps forward.

For over 60 years there were no newly approved therapies for MG by the FDA. That all changed in 2017 with the FDA's approval of Soliris. Since 2017, we have been on an exciting and almost constant track of approvals of new therapies. Most recently the FDA approved the treatment of Vyvgart and Vyvgart Hytrulo for all subtypes of MG, a landbreaking milestone.

With all certainty, I can share when I entered my role over 8 years ago, I had no idea what all of this meant. But today, it brings a much different meaning to me. In fact, after a recent webinar on advances with CAR-T presented by Mazen Dimachkie, MD we surveyed participants on how the information made them feel about the future of treatments with MG. Words such as "hope," "promising", "cure" and "amazing". Those are words I feel we can tuck in our pockets as we navigate our days with MG. There IS hope for the future and clinical trials offer us an opportunity to lay the groundwork.

We hope you enjoy this special edition "Clinical Trials" newsletter. We'd love to hear from you. Drop us a line at info@mgakc.org

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Two targeted treatments for adults with **generalized myasthenia gravis (gMG)**

UCB is committed to making a difference for people living with gMG by providing two treatment options

Is it time to rethink your current gMG therapy?

For people with gMG, symptoms present themselves differently in each diagnosed individual. While most people have fluctuating muscle weakness and fatigue, these symptoms vary from person to person and can range from mild to severe.

UCB offers two treatments that target different aspects of immune function related to gMG and have different methods of administration.

Scan to explore two distinct treatments that allow you and your doctor to choose the gMG treatment that fits your needs.

UCB OFFERS TWO TREATMENTS THAT TARGET gMG DIFFERENTLY:

An FcRn blocker

Harmful antibodies that cause gMG may stay in your body longer because of FcRn (neonatal Fc receptor).

A C5 inhibitor

Harmful anti-AChR antibodies activate part of your immune system called "complement," which works to attack your muscle cells.

Targeted treatments. More options.

Ask your doctor how a targeted therapy may help you meet your gMG treatment goals.

Learn more at **UCBforGMG.com**

Inspired by **patients.**
Driven by **science.**

FDA APPROVES VYVGART
for all subtypes of MG

On Friday May 8, 2026, the FDA announced the decision to approve the use of efgartigimod (Vyvgart) and Vyvgart Hytrulo for all subtypes of myasthenia gravis. What that essentially means is that not only is the use approved for individuals who are AChR positive but also for individuals who are MuSK+, seronegative and LRP4. This is a groundbreaking decision for the MG community and the first of it's kind.

The results from the Adapt Seron clinical trial showed positive results for all subtypes of MG. From the argenx press release, "The approval is based on data from the Phase 3 ADAPT SERON study, the largest study to date of patients with gMG who do not have detectable anti-acetylcholine receptor antibodies (AChR-Ab) across three serotypes – anti-MuSK-Ab positive, anti-LRP4-Ab positive, and triple seronegative. The overall population of patients in the study treated with VYVGART showed rapid, significant and sustained improvements in their gMG symptoms, including speech, vision, physical function and swallowing, among others. In addition, VYVGART was well tolerated across serotypes, with safety consistent with the established profile in patients with anti-AChR-Ab positive gMG."

Additional Detailed results from the Phase 3 ADAPT SERON study included in the press release:

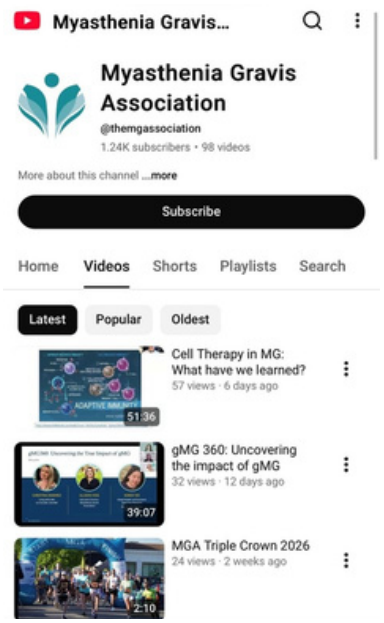
- Patients showed clinically meaningful improvements in disease activity across all three serotypes – anti-MuSK-Ab positive, anti-LRP4-Ab positive, and triple seronegative.
- The primary endpoint was met (p=0.0068), demonstrating that patients treated with VYVGART achieved a statistically significant improvement in MG-ADL (Myasthenia Gravis Activities of Daily Living) total score compared to placebo at week 4.
- In the overall population – across all serotypes in the study – mean change from baseline in patients treated with VYVGART was a clinically meaningful 3.35-point improvement in MG-ADL total score at week 4.
- Improvements in MG-ADL and Quantitative Myasthenia Gravis (QMG) scores were observed across subsequent treatment cycles in the overall population and in all serotypes studied.
- VYVGART was well tolerated across serotypes, with safety consistent with the established profile in patients with anti-AChR-Ab positive gMG.

VYVGART is available to patients in three administration options, including VYVGART Hytrulo self-injection with a prefilled syringe.

Did you know
Clincial Trial Education on our YouTube Channel?

Have you visited MGA's YouTube channel lately?
It's packed with educational webinars, personal stories from the MG community, and updates on treatment options and advocacy efforts. Be sure to head on over to our YouTube channel and hit subscibe to get updates on all of our videos.

SUBSCRIBE https://www.youtube.com/@themgassociation



NOW ENROLLING

Clinical Trial for People Living With Generalized Myasthenia Gravis

A Phase 3, Global, Open Label, Randomized Study of Miv-cel, a Chimeric Antigen Receptor (CAR) T-Cell Therapy Versus Standard of Care Immunotherapy

KYSA-6

What is myasthenia gravis?

Myasthenia gravis (MG) is a condition that causes muscle weakness and fatigue. It can affect only the eyes (ocular MG) or other muscles, including those used for movement, speaking and breathing (generalized MG). Most people with ocular MG develop generalized MG within 2 years.

What is a phase 3 clinical trial?

A phase 3 clinical trial compares a treatment's effectiveness against a control, such as current standard of care treatment, with further evaluation of its safety.

What is CAR T-cell therapy?

Miv-cel (mivocabtagene autoleucel, formerly KYV-101) is an investigational CAR T-cell therapy, a type of immunotherapy that is different than current treatments for generalized MG. **Miv-cel is made from a person's own immune cells to fight disease**.

Miv-cel is **administered one time** and works by eliminating B cells, which make antibodies—including the harmful ones that drive inflammation and symptoms in MG.

Prior to this study, miv-cel has been used to treat people with generalized MG and other autoimmune diseases under a form of compassionate-use treatment.

About KYSA-6

We are expanding our existing KYSA-6 phase 2 trial to include the phase 3 portion, which will evaluate miv-cel versus standard of care* treatment in adults with generalized MG. The primary objective of this phase 3 study is to establish miv-cel efficacy. The phase 2 portion of the KYSA-6 study is fully enrolled.

To learn more about this trial, scan the QR code or email
ClinicalTrialsInfo@kyvernatx.com

kyverna

Miv-cel is an investigational therapy.

*Standard of care may consist of traditional agents for MG (eg, prednisone, azathioprine, mycophenolate, methotrexate, chronic IVIg/PLEX) or complement pathway inhibitors (eg, eculizumab, ravulizumab). The following treatments are not allowed before screening: neonatal Fc receptor(FcRn) inhibitors within 4 weeks, or any other anti-CD20 or CD19 monoclonal antibody within 12 weeks.

What will the KYSA-6 study involve?

SCREENING

You will undergo assessments with study doctors to confirm if you are eligible

APHERESIS & CAR T CELL PREPARATION

Your T cells will be collected through a 2–4-hour blood draw procedure, called apheresis, and made into miv-cel. You'll receive standard of care treatment for 2 months while miv-cel is prepared

1 : 1 RANDOMIZATION

You will be assigned randomly to receive either miv-cel or continue standard of care

Miv-cel

Preparation for Miv-cel
About 1 week before infusion, you will receive a treatment called lymphodepletion to help prepare your immune system

Miv-cel Infusion
You will receive your miv-cel via a single infusion

Hospital Monitoring
You will stay at the hospital for 10-days to monitor your response and any potential side effects after miv-cel infusion

STANDARD OF CARE

You will continue to receive standard of care until the primary study evaluation at 24 weeks

Miv-cel CROSSOVER

You will have the option to crossover and receive miv-cel treatment

FOLLOW-UP | You will continue follow-up visits for 18 months after randomization, when the trial ends

CAR T-cell therapies can be associated with cytokine release syndrome (CRS) and immune effector cell-associated neurotoxicity syndrome (ICANS), which may be potentially serious or life-threatening but generally resolve within the first month after treatment.

- Symptoms of CRS include fever, nausea, feeling tired (fatigue), and body aches and can progress in severity and may include low blood pressure, shock, and potentially organ failure.
- Symptoms of ICANS include fatigue, uncontrolled movements (tremors), impairment in thinking, loss of speech, muscle weakness, or more severe symptoms such as seizures and swelling in the brain.

To learn more about this trial, scan the QR code or email:
ClinicalTrialsInfo@kyvernatx.com

kyverna

Miv-cel is an investigational therapy.

UNDERSTANDING MYASTHENIA GRAVIS CLINICAL TRIALS

COMMON QUESTIONS

Why Are Clinical Trials Important for MG?

Clinical trials are how researchers learn:

- Whether a treatment works
- How safe it is
- What side effects may occur
- Which groups of patients benefit most

The MG treatment landscape has changed dramatically in recent years because of clinical research. New targeted therapies have created additional options for many patients, particularly those with generalized MG. Without clinical trials, these advancements would not happen.

How Do I Know If I Qualify?

Every trial has eligibility criteria, sometimes called "inclusion" and "exclusion" criteria. These requirements help researchers study treatments safely and accurately.

In MG clinical trials, eligibility may depend on:

- MG subtype
- Antibody status (AChR antibody-positive, MuSK antibody-positive, LRP4 antibody-positive, and Seronegative)
- Disease severity as defined by MGFA classification system
- Current medications
- Age
- Previous treatments
- Overall health

For example, some MG trials are only open to:

- AChR antibody-positive individuals
- MuSK-positive individuals
- Seronegative individuals
- Generalized MG patients
- Juvenile MG patients

Some studies may also require:

- Stable background therapy
- Specific MG-ADL scores
- Vaccination requirements
- Certain symptom severity levels

This is why two people with MG may qualify for completely different studies.

What Are the Different Phases of Clinical Trials?

Clinical trials happen in phases, with each phase answering different questions.

Phase 1

- Small number of participants
- Focuses mainly on safety and dosage
- Researchers identify side effects and safe dosing ranges

Phase 2

- Larger participant group
- Evaluates whether the treatment appears effective
- Continues monitoring safety

Phase 3

- Often involves hundreds or thousands of participants
- Compares the treatment to standard care or placebo
- Helps determine whether the treatment should seek FDA approval

Phase 4

- Happens after FDA approval
- Monitors long-term safety and effectiveness in broader populations

How Do I Find MG Clinical Trials?

Trusted resources include:

- ClinicalTrials.gov
- MGNet Clinical Trial Resources
- Myasthenia Gravis Association Clinical Trials Page

UNDERSTANDING MYASTHENIA GRAVIS CLINICAL TRIALS

COMMON QUESTIONS CONTINUED

What is an MG Subtype?

Myasthenia gravis (MG) is not a one-size-fits-all condition. While people often hear "MG" as a single diagnosis, it actually includes several subtypes that help describe what is driving the disease and how it may show up in the body.

These subtypes are usually based on which antibodies are involved (or not involved) and can help guide treatment decisions, prognosis, and eligibility for certain clinical trials.

Antibody-positive MG subtypes

Most people with MG fall into one of these antibody-based categories:

- **AChR antibody-positive MG** (Acetylcholine receptor) This is the most common subtype. The immune system targets acetylcholine receptors at the neuromuscular junction, making it harder for nerves to activate muscles normally.
- **MuSK antibody-positive MG** (Muscle-specific kinase) A less common subtype that often affects bulbar muscles (speech, swallowing, facial muscles) more prominently. Symptoms can sometimes progress more quickly or respond differently to standard treatments.
- **LRP4 antibody-positive MG** (Lipoprotein receptor-related protein 4) A rarer subtype that is still being studied. It may present similarly to other generalized forms of MG but with different immune mechanisms.

Seronegative MG

Some people have clear MG symptoms but test negative for the commonly known antibodies. This is called seronegative MG.

In some cases, newer or less commonly tested antibodies may later be identified. In others, the exact immune target may still be unknown, but the diagnosis is made based on clinical symptoms and testing like EMG or response to treatment.

Other ways MG is described

In addition to antibody type, MG subtypes may also be described by:

- **Ocular MG** – symptoms are limited to the eyes (drooping eyelids, double vision)
- **Generalized MG** – symptoms affect multiple muscle groups beyond the eyes
- **Early-onset** vs. **late-onset MG** – based on age at diagnosis
- **Thymoma-associated MG** – when MG occurs alongside a tumor of the thymus gland

Why MG Subtypes Matter

Understanding MG subtypes helps:

- Guide treatment choices and expectations
- Identify which therapies may work best for a person's specific immune profile
- Determine eligibility for clinical trials
- Improve research into more targeted treatments
- While every person's experience with Myasthenia Gravis is unique, these subtypes help providers better understand what is happening at an immune level—and how to tailor care more effectively.

Explore the only FDA-approved gMG treatment given 2x a year

SCAN TO LEARN MORE

gMG, generalized myasthenia gravis.

AMGEN

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UNDERSTANDING MYASTHENIA GRAVIS CLINICAL TRIALS

COMMON QUESTIONS CONTINUED

What is the MGFA Classification System?

When talking about myasthenia gravis (MG), you may hear providers or researchers refer to something called the MGFA Clinical Classification system. MGFA stands for the Myasthenia Gravis Foundation of America, and this system is used to help describe how MG is affecting the body at a given point in time.

Think of it as a shared "language" clinicians use to communicate disease severity. While MG symptoms can fluctuate day to day, the MGFA classification gives a snapshot of how mild or advanced symptoms are overall and which parts of the body are involved.

Why this Classification System Matters

The MGFA system is commonly used in clinic settings and research to:

- Help providers clearly communicate disease severity
- Track how MG is progressing or responding to treatment over time
- Support treatment planning and clinical decision-making
- Define eligibility and outcomes in clinical trials

While it doesn't capture every detail of how MG impacts someone day-to-day, it works alongside tools like the MG-ADL scale to give a more complete picture of both clinical severity and lived experience.

What Is MG-ADL?

MG-ADL stands for the Myasthenia Gravis Activities of Daily Living scale. It is a common tool used in clinical trials to measure how MG symptoms affect daily functioning.

Questions often focus on:

- Speaking
- Swallowing
- Breathing
- Chewing
- Brushing hair or teeth
- Rising from a chair
- Double vision
- Eyelid drooping

Researchers use MG-ADL scores to track improvement or worsening symptoms over time.

MGFA Clinical Classes

- **Class I** – Ocular MG Symptoms are limited to the eyes only. This includes things like drooping eyelids (ptosis) and/or double vision (diplopia). No other muscle groups are affected.
- **Class II** – Mild Generalized MG Mild weakness beyond the eyes. This may involve the face, arms, legs, or swallowing muscles, but symptoms are still considered mild.
  - **Class IIa**: Mainly limb or body muscle involvement
  - **Class IIb**: Mainly swallowing, speech, or breathing muscles
- **Class III** – Moderate Generalized MG More noticeable weakness affecting multiple muscle groups and beginning to impact daily activities.
  - **Class IIIa**: Predominantly limb or body muscles
  - **Class IIIb**: Predominantly swallowing, speech, or breathing muscles
- **Class IV** – Severe Generalized MG Significant weakness that can interfere with basic daily function and independence.
  - **Class IVa**: Limb/body muscles are most affected
  - **Class IVb**: Swallowing, speech, or breathing muscles are most affected
- **Class V** – Myasthenic Crisis This is the most severe category and is considered a medical emergency. It involves respiratory muscle weakness that requires intubation with or without mechanical ventilation.

UNDERSTANDING MYASTHENIA GRAVIS CLINICAL TRIALS

COMMON QUESTIONS CONTINUED

Will I Receive a Placebo?

Possibly — but not always.
A placebo is an inactive substance that looks like the study treatment.
Placebos help researchers compare whether a treatment is truly effective.

However, many MG trials:

- Continue standard-of-care treatment alongside the study drug
- Use open-label extensions after placebo-controlled portions
- Allow participants access to the active treatment later

The informed consent process should clearly explain:

- Whether placebo is involved
- Your chances of receiving it
- What treatments you can continue during the trial

Is Participating Safe?

Clinical trials carry risks, but participant safety is heavily monitored.

Before joining a study, participants go through an informed consent process that explains:

- Potential risks
- Side effects
- Study procedures
- Emergency protocols
- Participant rights

You can leave a clinical trial at any time.

Safety is monitored throughout the study by:

- Research teams
- Ethics committees
- Regulatory agencies
- Data safety monitoring boards

What Are the Possible Benefits?

Potential benefits may include:

- Access to emerging therapies
- Closer medical monitoring
- Helping advance future MG treatments
- Contributing to scientific understanding

However, clinical trials cannot guarantee improvement.

Some participants experience benefits, while others may not respond to the treatment.

What Are the Risks?

Possible risks may include:

- Side effects
- Treatment not working
- Additional appointments or procedures
- Fatigue from travel or testing
- Emotional stress or uncertainty

Because MG symptoms can fluctuate, it is especially important for participants to discuss risks carefully with their neurologist.

Will Participation Affect My Daily Life?

Every study is different.

Some trials may involve:

- Frequent clinic visits
- Lab work
- Infusions or injections
- Symptom tracking
- Travel to specialty centers

Others may be much less intensive.

Questions worth asking include:

- How often are visits?
- Is travel reimbursement available?
- How long is the study?
- Can I continue working or attending school?
- Can caregivers attend appointments?

UNDERSTANDING MYASTHENIA GRAVIS CLINICAL TRIALS

COMMON QUESTIONS CONTINUED

Are Clinical Trials Free?

Typically, the study treatment and research-related procedures are covered by the sponsor of the clinical trial.

Some studies also reimburse:

- Travel
- Hotels
- Meals
- Parking

However, standard medical care unrelated to the study may still go through insurance.Always ask for clarification before enrolling.

Clinical trials are deeply personal decisions. Some people feel excited by the opportunity to contribute to research and access new therapies. Others may decide that a trial is not the right fit for them right now. Both choices are valid.

The most important thing is having clear, accurate information so you can make informed decisions alongside your healthcare team.

Questions to Ask Before Joining a Trial

If you are considering participation, it may help to ask:

- What is the purpose of this study?
- Why do researchers think this treatment may help?
- What are the risks and possible side effects?
- Will I continue my current medications?
- Is there a placebo?
- What happens if my symptoms worsen?
- How much time is required?
- What happens when the trial ends?

As MG research continues to evolve, clinical trials remain one of the most important ways the community moves toward better treatments, improved quality of life, and future breakthroughs.

MGA SUPPORTS CLINICAL TRIALS MODERNIZATION ACT

In August 2025, the Myathenia Gravis Association (MGA) along with 131 other patient advocacy organizations signed on to the S. 4440, Clincial Trial Modernization Act which was recently intoduced by Sens. Tim Scott (R-SC) and Mark Warner (D-VA). You can view their announcement and bill text here: press release.

This bill would increase access to clinical trials for underrepresented communities such as those in rural areas and/or those who are low income, reduce bureaucratic red tape, and enhance U.S. innovation and leadership in developing new treatments and therapies.

The Clinical Trials Modernization Act would address barriers across the patient journey by:

- ·Supporting enrollment among historically underrepresented populations;
- ·Allowing coverage of essential participation costs (food, transportation, lodging);
- ·Permitting payment of copays and coinsurance tied to trial participation;
- ·Enabling remote monitoring, reducing unnecessary travel; and
- ·Making up to $2,000 in trial payments tax-exempt while preserving safety net program eligibility.

Improving legislation for clinical trials is vital to the success for not only the patient community but to clinicians and industry.

The SYNAPSE-MG Clinical trial
for generalized Myasthenia Gravis (gMG)

An investigational study evaluating a **potential new oral treatment for MG** that allows participants to remain on their current medications.*

Who's eligible?

Thosewith a confirmed diagnosis of gMG with AChR or MuSK autoantibodies, along with other key eligibility requirements:

- **18–75 years old**
- **Able to take oral tablets**
- **Able to attend in-person clinic visits over an 8-week time period**

NMD PHARMA® NEUROMUSCULAR DISORDERS | CLINICAL TRIALS

*For many patients, most gMG medications can be continued exactly as they are currently being taken

For more information about the clinical trial scan the QR code or visit NMD Pharma's Clinical Trial website

nmdclinicaltrials.com/clinical-trials/synapse-mg/

MGNET & RARE DISEASE NETWORK

DEDICATED TO MYASTHENIA GRAVIS

The Rare Disease Network dedicated to Myasthenia Gravis, MGNet, is one of about 20 rare disease research groups supported by the National Institutes of Health (NIH). About 20 years ago, the NIH created this program to enhance development of treatments for rare diseases. The motivation was that research for rare disease comes with shared challenges.

First, rare diseases affect only a small number of people, which makes their comprehensive study by a single research group extremely difficult.

Second, many of these diseases did not have clear ways to measure how severe symptoms are in a rigorous manner. Without good measurements, it is hard to run clinical trials that can assess whether a treatment works. In addition to clinical exams, researchers also saw the need for biological markers. These markers can help diagnose disease, track how severe it is, and predict which treatments may work best for each patient.

The MGNet was first funded in 2019 and was renewed last year for another five-year period. In its first five years, the network created one of the largest collections of patient samples for myasthenia gravis. During the COVID-19 pandemic, we also developed new ways to monitor patients using telemedicine. These tools have grown into platforms that computer vision and artificial intelligence to improve the examination.

We have also made strong progress in understanding how antibodies damage the neuromuscular junction. Using advanced methods, we have identified biological markers that may help predict poor response to treatments.

In the next five years, we plan to build new ways to collect high-quality research data during routine clinic visits and thereby understand the great variation how MG and its treatments effect patients. We will study “biological age” in MG and whether this impacts treatment response or development of MG. Biological age is a relatively new concept in medicine that is a better predictor of health than chronological age. We will also continue to study how acetylcholine receptor antibodies cause damage, with the goal of improving treatment.

We also support the next generation of researchers by providing training and small grants to help them start new projects. In the first five years, we funded investigators in Italy, Germany, and the United States. We are continuing this effort by supporting new researchers at the University of Chicago and Yale University. These programs are supported by funds from the NIH, Conquer MG, MGA of Michigan and the MG Association. Argenx also provides unrestricted funds. A new funder of our research is the International Thymic Malignancy Interest Group.

Through collaboration across several academic centers and our collaborations, we aim to speed up discovery and improve care for patients.

Living with **ocular** (eye-only) **myasthenia gravis?**

We seek the support of people living with **ocular (eye-only) myasthenia gravis** for the **MyVision study.**

We also understand that some people living with ocular myasthenia gravis (oMG) may have someone supporting them, so if you or someone you know has oMG, we would like to hear from you.

Ocular myasthenia gravis is a condition that can cause double vision, droopy eyelids, fatigue, light sensitivity, or difficulty looking in different directions. These symptoms can make everyday activities challenging like driving, reading, and watching television, even if you are already on treatment.

The MyVision study is testing a study drug to see the effects on eye-only symptoms in adults with ocular myasthenia gravis (oMG). The study is hoping to better understand oMG and its symptoms while advancing scientific research at the same time. The aim of the study is to learn how well the study drug works (efficacy) and what side effects people experience while taking it (safety).

You may be able to join the MyVision study if you:

- Are at least 18 years of age
- Have been diagnosed with oMG by a doctor
- Are **not** experiencing weakness in other muscles in your body such as your face, throat, arms, and/or legs

There are other requirements for taking part in this study, which the study team will discuss with you.

If you qualify and decide to join, you will get:

- The study drug or placebo (a placebo is similar to the study drug but contains no medicinally active ingredient)
- Close medical care and follow-up throughout the study

Reimbursement for travel and other study-related expenses may be provided.

There is an extension study called MyVisionXT that MyVision participants may have an opportunity to join. In the MyVisionXT study, all participants will receive the study drug—there is no chance of receiving placebo.

If you are interested in taking part in the MyVision study, please visit myvisionstudy.com or contact the study team to learn more:

MyVision

Inspired by patients. Driven by science.

MG0038_Flyer_v1.0_US Eng_15JAN2026

MGA FUNDS RESEARCH AT 2 INSTITUTIONS

The MGA was proud to support clinical research for Myasthenia Graivs (MG) at two institutions. A $50,000 reearch grant was given to the Neurology Department at the Univerity of Kansas Medical Center anda $25,000 research grant was given to the MGnet at George Washington University in 2025. The MGA is proud to support the research intiatives that are ongoing at each institution. This was a 67% increase in research support year over year.

Left: Pictured with members of the Neurology department at the University of Kansas in Kansas City.

Right: Pictured with Dr. Henry Kaminiski and the MGnet.

Spotlight on Research Institutions

MYASTHENIA CLINICAL TRIALS

An update from the Neuromuscular Research Division at the University of Kansas Medical Center

Contributed by Constantine Farmakidis, MD, Associate Professor, Neurology, University of Kansas Medical Center and Andrew Heim, Research Project Manager, Neuromuscular Research Division University of Kansas Medical Center

The KU neuromuscular research division has been a leading clinical research site for myasthenia gravis and has participated in most of the clinical trials for the wave of new MG treatments approved during the last decade. The key has been teamwork between the MG patient community, our ultra-professional research team, the neuromuscular physician team, and of course, patient advocacy organizations like the MGA.

As of the present, it is hard to overstate how much progress has been made in myasthenia treatment in recent years. The work continues however, with many highly promising treatments undergoing clinical trials and our research division is continuing to lead as one of the major centers in myasthenia gravis clinical research in North America. What follows are short summaries of ongoing and soon to start myasthenia clinical trials. Please read and contact us with questions or if you are interested in participating. We look forward to answering your questions.

Pharmaceutical Company Initiated Clinical Trials

ARGX-113-PASS-2208
Study sponsor: Argenx
Site principal investigator: Dr. Mamatha Pasnoor
Investigational agent: efgartigimod (an already FDA approved drug for acetylcholine receptor antibody positive MG)
Mechanism of action: reduces levels of IgG antibodies, including the acetylcholine receptor antibody which is known to cause myasthenia gravis
How is it given: infusion in the veins or injection underneath the skin
Major inclusion requirements: autoantibodies for AChR
The purpose of this study is to carefully study the long-term safety of efgartigimod in patients with generalized myasthenia gravis, including the risk of infection. As you may know efgartigimod is the generic name for Vyvgart, and this drug has been approved for MG since 2021. Individuals eligible for recruitment are those expected to start treatment with efgartigimod or who are within the first cycle of treatment at enrollment. In the control group that will also be studied, individuals should not have prior efgartigimod exposure, and not be planned to start the drug.

RESET-MG CAB-201-004
Study sponsor: Cabaletta Bio
Site principal investigator: Dr. Mazen Dimachkie
Investigational agent: genetically modified T-cells, or CART cells, a live, cell-based therapy
Mechanism of action: genetically modified T-cells, or CART cell, are retrained to eliminate CD-19 positive B-cells through the body. The B-cells produce antibodies including the antibodies that in error cause myasthenia
How is it given: CART cell infusion through the veins and additional medications for preparation
Major inclusion requirements: autoantibodies for AChR, MuSK and/or LRP4 or seronegative MG
Genetically modified T-cells have been used successfully to in treatment-resistant blood cancers. This is a process where your T-cells are removed from your own body, genetically reprogrammed to fight cancer, and then placed back into the body to fight the cancer. The same approach is used in this myasthenia trial and the goal here is to use genetically reprogrammed T-cells to institute a deep reboot of the immune system, with a goal of achieving a long lasting and deep remission of myasthenia.

Descartes-08 mRNA CAR-T Cell Therapy in Myasthenia Gravis (AURORA)
Study sponsor: Cartesian Therapeutics
Site principal investigator: Dr. Mamatha Pasnoor
Investigational agent: genetically modified T-cells, or CART cells
Mechanism of action: CART cells, are genetically retrained to eliminate BCMA-positive B-cells through the body. These B-cells produce antibodies including the inappropriately produced antibodies that in error cause myasthenia gravis
How is it given: infusion in the veins
Major inclusion requirements: autoantibodies for AChR
This is another CART based clinical trial. Once again, genetically modified T-cells are reprogrammed to institute a deep reboot of the immune system, with a goal of achieving a long lasting and deep remission of myasthenia. In the Cabaletta Bio study above, the target is CD-19 positive B-cells, where in this clinical trial (Descartes-08 mRNA) the target is BCMA-positive B cells. Another key difference is that Cabeletta Bio uses DNA to reprogram T-cells into CART cells, whereas Decartes-08 uses mRNA. Please reach out with questions with these very interesting CART studies.

Oral Cladribine Compared with Placebo in Participants with Myasthenia Gravis (MyClad)
Study sponsor: Merck Healthcare KGaA
Site principal investigator: Dr. Mamatha Pasnoor
Investigational agent: cladribine
Mechanism of action: cladribine inhibits DNA synthesis in rapidly proliferating cell, like B cells and to a lesser degree T cells. Both of these immune system cells have a key role in myasthenia gravis, and inhibiting their development could help control myasthenia gravis disease activity
How is it given: tablet taken by mouth
Major inclusion requirements: positive antibodies against AChR or MuSK
This is later stage and larger, or phase 3 clinical trial. Very notably this drug is already FDA approved for multiple sclerosis, and thus has already cleared a major test of safety and tolerability. Also very notably, cladribine is an oral tablet, which potentially in the future could make for the first disease modifying therapy for myasthenia that is taken by mouth and is approved by the FDA (prednisone, azathioprine, mycophenolate are not FDA approved for MG).

Povetacicept in Adults with Generalized Myasthenia Gravis (ETNA)
Study sponsor: Vertex Pharmaceuticals Incorporated
Site principal investigator: Dr. Constantine Farmakidis
Investigational agent: povetacecept
Mechanism of action: povetacecept is an Fc fusion protein that targets BAFF and APRIL signaling molecules that help B-cells survive, develop, and proliferate. Recall that B-cells produce all antibodies, including the inappropriately produced antibodies that in error cause MG. So, the concept here is to interfere with the development of B-cells, and have fewer of the antibodies that cause myasthenia gravis.
How is it given: injection under the skin
Major inclusion requirements: positive antibodies against AChR or MuSK
This is a phase 2 clinical trial aiming to evaluate the safety, tolerability and potential for clinical benefit in myasthenia gravis. There is already a body of evidence that indicates that BAFF-APRIL inhibitors can successfully treat myasthenia and can be well tolerated. Povetacecept could potentially provide another option for patients in the BAFF-APRIL category of drugs.

Telitacicept for the Treatment of Generalized Myasthenia Gravis (UPSTREAM MG)
Study sponsor: Vor Biopharma
Site principal investigator: Dr. Mamatha Pasnoor
Investigational agent: telitacicept

Telitacicept for the Treatment of Generalized Myasthenia Gravis (UPSTREAM MG) Continued
Mechanism of action: telitacicept is a fully human fusion protein that targets and BAFF and APRIL signaling molecules that help B-cells survive, develop, and proliferate. Recall that B-cells produce all antibodies, including the inappropriately produced antibodies that cause MG. So, the concept here is to interfere with the development of B-cells, and have fewer of the antibodies that cause myasthenia gravis.
How is it given: injection under the skin
Major inclusion requirements: positive antibodies against AChR or MuSK
This is a later stage and larger, or phase 3 clinical trial. There is already a body of evidence that indicates that telitacicept can treat myasthenia. And this clinical trial aims to test if this first in class BAFF-APRIL inhibitor agent, can be confirmed to be effective and safe in a larger global clinical trial.

Investigator Initiated Clinical Trials

The Neuromuscular Research Division at KUMC is regularly involved in academic research projects, meaning the clinical trial is led by a clinical researcher at an academic medical center, like KUMC. The money to pay for this research can come from the other NIH, other organizations that support medical research or through a partnership with a pharmaceutical company that has money to pay for the project. Our team is currently involved in two academic-pharmaceutical company partnership projects in myasthenia gravis.

The first is led by Dr. Miriam Freimer at Ohio State University in partnership with UCB. UCB is a pharmaceutical company and the manufacturer of zilucoplan (ZILBRYSQ), which was approved for the treatment of myasthenia gravis in 2023. This project will seek to test whether zilucoplan is an effective treatment for hospitalized patients with severe exacerbations, and whether it could be effective alternative to plasma exchange or IVIG that are now the standard of care when very fast and effective treatment is needed to rescue patients from very severe myasthenia weakness.

The second project is led by Dr. Ali Habib at the University of California, Irvine in partnership with Nkarta, Inc. Nkarta is a biotechnology company working to apply innovative cell-based therapies to improve treatment of autoimmune diseases. So far you may have become aware of experimental treatments that seek to change the genetic makeup of T-cells and train them to aggressively treat myasthenia gravis. In this project, the research team seeks to develop the biotechnology to change the genetic makeup of T-cells and retrain them to treat myasthenia gravis.

Explore CLINICALTRIALS.GOV Today

Did you know that legally ever clinical trial has to be listed on Clinicaltrials.gov? Scan the QR code and you can search for clinical trials associated with myasthenia gravis and in a particular or desired location.

CAN YOU MAKE AN IMPACT IN MYASTHENIA GRAVIS (MG) RESEARCH?

MYCLAD

The MyClad Study is researching an investigational medication taken by mouth for generalized myasthenia gravis (gMG) and is now enrolling. Join us.

The MyClad Study is looking to learn more about an investigational medication taken by mouth regarding its safety and ability to treat people with gMG. The investigational medication will be given as a capsule to be swallowed. We want to evaluate the ability to improve your gMG symptoms when using the investigational medication.

You may be eligible for this study if you:

- are an adult of at least 18 years of age or older
- have a confirmed diagnosis of MG with generalized muscle weakness.

Your doctor will review the study requirements and discuss your eligibility with you.

You will receive all study-related procedures and the investigational medication at no cost.

Study participation will last for about 3 years.

This study has been designed with gMG patients' feedback and reviewed by an Institutional Review Board (IRB)/Ethics Committee (EC), which protects the rights, safety, and well-being of the participants.

To learn more, ask your doctor about the MyClad Study or scan the QR code.

mycladstudy.com

EMD SERONO

Sponsored Content, 05 Sep 2025 [VDI USA(en)]

Clinical Trial Terms

This Glossary of Terms is sourced from ClinicalTrials.gov, learn more from their glossary at https://clinicaltrials.gov/study-basics/glossary

A

Active comparator arm is an arm type in which a group of participants receives an intervention/treatment considered to be effective (or active) by health care providers.
Arm is a group or subgroup of participants in a clinical trial that receives a specific intervention/treatment, or no intervention, according to the trial's protocol.
Arm type is a general description of the clinical trial arm. It identifies the role of the intervention that participants receive. Types of arms include experimental arm, active comparator arm, placebo comparator arm, sham comparator arm, and no intervention arm.

C

Clinical study is a research study involving human volunteers (also called participants) that is intended to add to medical knowledge. There are two types of clinical studies: interventional studies (also called clinical trials) and observational studies.
Cross-over assignment is a type of intervention model describing a clinical trial in which groups of participants receive two or more interventions in a specific order. For example, two-by-two cross-over assignment involves two groups of participants. One group receives drug A during the initial phase of the trial, followed by drug B during a later phase. The other group receives drug B during the initial phase, followed by drug A. So during the trial, participants "cross over" to the other drug.

E

Early Phase 1 (formerly listed as Phase 0) is a phase of research used to describe exploratory trials conducted before traditional phase 1 trials to investigate how or whether a drug affects the body. They involve very limited human exposure to the drug and have no therapeutic or diagnostic goals (for example, screening studies, microdose studies).
Expanded access provides a way for patients with serious diseases or conditions who cannot participate in a clinical trial to gain access to a medical product that has not been approved by the U.S. Food and Drug Administration (FDA). Also called compassionate use.
Experimental arm is an arm type in which a group of participants receives the intervention/treatment that is the focus of the clinical trial.

I

Intervention model refers to the general design of the strategy for assigning interventions to participants in a clinical study. Types of intervention models include: single group assignment, parallel assignment, cross-over assignment, and factorial assignment.
Interventional study (clinical trial) is a type of clinical study in which participants are assigned to groups that receive one or more intervention/treatment (or no intervention) so that researchers can evaluate the effects of the interventions on biomedical or health-related outcomes. The assignments are determined by the study's protocol. Participants may receive diagnostic, therapeutic, or other types of interventions.

M

Masking refers to a clinical trial design strategy in which one or more parties involved in the trial, such as the investigator or participants, do not know which participants have been assigned which interventions. Types of masking include: open label, single blind masking, and double-blind masking.

N

NCT number is an unique identification code given to each clinical study record registered on ClinicalTrials.gov. The format is "NCT" followed by an 8-digit number (for example, NCT00000419). Also called the ClinicalTrials.gov identifier.
No intervention arm is an arm type in which a group of participants does not receive any intervention/treatment during the clinical trial.

Clinical Trial Terms

This Glossary of Terms is sourced from ClinicalTrials.gov, learn more from their glossary at https://clinicaltrials.gov/study-basics/glossary

P

Parallel assignment refers to a type of intervention model describing a clinical trial in which two or more groups of participants receive different interventions. For example, a two-arm parallel assignment involves two groups of participants. One group receives drug A, and the other group receives drug B. So during the trial, participants in one group receive drug A "in parallel" to participants in the other group, who receive drug B.

Participant flow is a summary of the progress of participants through each stage of a clinical study, by study arm or group/cohort. This includes the number of participants who started, completed, and dropped out of the study.

Phase 1 is a phase of research that describes clinical trials that focus on the safety of a drug. They are usually conducted with healthy volunteers, and the goal is to determine the drug's most frequent and serious adverse events and, often, how the drug is broken down and excreted by the body.

Phase 2 is a phase of research that describes clinical trials that gather preliminary data on whether a drug works in people who have a certain condition/disease (the drug's effectiveness). For example, participants receiving the drug may be compared to similar participants receiving a different treatment, usually an inactive substance (called a placebo) or a different drug. Safety continues to be evaluated, and short-term adverse events are studied.

Phase 3 is a phase of research that describes clinical trials that gather more information about a drug's safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs. These studies typically involve more participants.

Phase 4 is a phase of research that describes clinical trials occurring after FDA has approved a drug for marketing. They include postmarket requirement and commitment studies that are required of or agreed to by the study sponsor. These trials gather additional information about a drug's safety, efficacy, or optimal use.

Phase Not Applicable describes trials without FDA-defined phases, including trials of devices or behavioral interventions.

Placebo is an inactive substance or treatment that looks the same as, and is given in the same way as, an active drug or intervention/treatment being studied.

R

Randomized allocation is a type of allocation strategy in which participants are assigned to the arms of a clinical trial by chance.

S

Secondary outcome measure in a clinical study's protocol is a planned outcome measure that is not as important as the primary outcome measure for evaluating the effect of an intervention but is still of interest. Most clinical studies have more than one secondary outcome measure.

Statistical analysis plan (SAP) is the written description of the statistical considerations and methods for analyzing the data collected in the clinical study.

U

U.S. Agency for Healthcare Research and Quality (AHRQ) is an agency within the U.S. Department of Health and Human Services. AHRQ's mission is to produce evidence to make health care safer, higher quality, more accessible, equitable, and affordable, and to work within the U.S. Department of Health and Human Services and with other partners to make sure that the evidence is understood and used.

U.S. Food and Drug Administration (FDA) is an agency within the U.S. Department of Health and Human Services. The FDA is responsible for protecting the public health by making sure that human and veterinary drugs, vaccines and other biological products, medical devices, the Nation's food supply, cosmetics, dietary supplements, and products that give off radiation are safe, effective, and secure.

adapt FORWARD
myasthenia gravis platform

Are you living with MG?

Find out if you qualify for one of the ADAPT FORWARD PLATFORM **clinical research studies** for adults living with Myasthenia Gravis (**MG**).

You may be eligible to participate if you are:

- At least 18 years old.
- Not pregnant, or actively trying to get pregnant.
- Diagnosed with MG.

Additional criteria to participate apply which the study doctor will discuss with you.

If you qualify, you will receive:

- Study-related exams and procedures at no cost.
- Study drug(s) at no cost.

You will be compensated for study-related expenses such as meals or travel. Travel arrangements may be provided.

Interested in learning more?

To learn more about the study or to find out if the study might be right for you or someone you know, please contact:

argenx
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or visit
w w w.clinicaltrials.argenx.com/adaptforward
for more information

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Are You Living With **Myasthenia Gravis (MG)?**

Explore whether the UPSTREAM MG Phase 3 Clinical Study is right for you

Thefocusof today'sMG treatmentisshiftingfromsymptomrelief to targeting the immune system, where the root cause lies.

This global study is testing a drug that targets the immune system to see what effect it may have on the daily lives of those who have generalized myasthenia gravis.

Our study drug will be administered as weekly injections, some of which may be self-injected (or given by a caregiver) at home between study visits.

For information about this study, including participation requirements, **email us at study@vorbio.com**

Please discuss study participation with your treating doctor.

For more about Vor Bio, scan here.

VOR

CAR-T THERAPY & MYASTHENIA GRAVIS (MG)

A NEW AND EMERGING TREATMENT APPROACH

Recent years have brought significant progress in understanding and treating Myasthenia Gravis (MG), particularly in the rapidly evolving field of cellular therapies.

What is CAR-T Therapy?

CAR-T (Chimeric Antigen Receptor T-cell therapy) is a treatment that is being studied in autoimmune diseases like MG. It is designed to "reprogram" a patient's own immune system, so it stops producing harmful antibodies. CAR-T therapy has been used successfully in some blood cancers, and researchers are now exploring how it may help "reset" the immune system in diseases like MG.

How CAR-T Works

CAR-T therapy involves several steps:

1. Collecting immune cells
   - A patient's T-cells (a type of white blood cell) are collected from the blood.
2. Reprogramming the cells
   - In a laboratory, the cells are modified to recognize and remove specific immune cells involved in antibody production.
3. Growing the cells
   - The modified cells are multiplied into large numbers.
4. Infusing the cells back
   - The updated cells are given back to the patient through an IV.
5. "Resetting" the immune system
   - These cells help reduce the immune cells that produce harmful MG antibodies, with the goal of allowing the immune system to restart in a healthier way.

What Does CAR-T Target in MG?

In Myasthenia Gravis, CAR-T therapy is designed to target the immune cells that help produce the harmful antibodies attacking the neuromuscular junction. Instead of broadly suppressing the immune system, CAR-T aims to more precisely target/eliminate the problem immune cells and allow healthier immune cells to regrow.

Researchers are studying targets such as:

- CD19 – found on many B cells that help produce antibodies
- BCMA – found on long-lived plasma cells that make antibodies

These immune cells are responsible for producing the antibodies that cause MG symptoms.

Who Might Consider CAR-T in MG?

At this time, CAR-T is:

- Only available through clinical trials
- Generally considered for people who:
  - Have moderate to severe MG
  - Have not responded well to standard treatments

Not typically used as a first treatment option

CAR-T THERAPY & MYASTHENIA GRAVIS (MG)

A NEW AND EMERGING TREATMENT APPROACH

Who Might Consider CAR-T in MG?

Potential Benefits

Early research in autoimmune diseases suggests CAR-T therapy may:

- Reduce or eliminate harmful antibody-producing cells
- Improve muscle strength and MG symptoms
- Potentially lead to long-term remission in some patients

However, it is still considered investigational (not yet approved for MG).

Possible Risks and Side Effects

Because CAR-T strongly affects the immune system, side effects can occur:

1. Cytokine Release Syndrome (CRS)
   - Fever, fatigue, low blood pressure
   - Flu-like symptoms
   - Can range from mild to serious
2. Neurologic Side Effects (ICANS)
   - Confusion
   - Difficulty speaking or concentrating
   - Headache or, rarely, seizures

Doctors closely monitor patients and have treatments to manage these reactions.

What Have Early Studies Shown?

In clinical research so far:

- Many patients improved in symptom severity
- Some reached "minimal symptom expression" (very few or no daily symptoms)
- Benefits were seen within months in some studies

CAR-T therapy is a promising new research approach that aims to reset the immune system in MG, but it is still experimental. More research is needed before it becomes widely available.

Where the Research Stands

- Early clinical trials in MG are showing promising results
- Both CD19- and BCMA-targeted therapies are being studied

Larger studies are still underway to confirm safety and effectiveness.

Why This Matters

Research in Myasthenia Gravis is rapidly advancing. New therapies are being studied that aim to more precisely target the immune system and reduce disease activity in people living with MG.

These treatments are still investigational (not yet widely available) but show promising early results.

Find A SUPPORT GROUP Near you

The Myasthenia Gravis Association (MGA) has support groups meeting in many areas. We have in person support groups in the midwest and virtual groups which are open to anybody, anywhere. We'd love to have you join us! In addition, we have groups that meet during the day, in the evening, on the weekend and everything in between! Scan the QR code and find which group will best meet your needs.

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Cut and enclose in envelope. Mail to MGA address below:

I want to support the MGA by becoming a 2026

- ☐ $25.00 Basic Member
- ☐ $66.00 64th Anniversary Member
- ☐ $100.00 Sustaining Member
- ☐ $500.00 Patron Member
- ☐ $1,000.00 Lifetime Member

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$_____In honor of: _____

Name :_____

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City, State, Zip:_____

Phone:_____

Email: _____

I am a: ☐ MG PATIENT ☐ RELATIVE ☐ FRIEND ☐ OTHER

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