Clinical Study Results

Inspired by patients. Driven by science.

Study Sponsor: UCB Biopharma SRL

Treatment Studied: Zilucoplan

Protocol Number: DV0013

Study Purpose: A study to learn if a zilucoplan auto-injector is safe and works in adults with myasthenia gravis

Thank you

UCB thanks all the participants of this study. All the participants helped the researchers learn more about using zilucoplan in people living with generalized myasthenia gravis.

This is a summary of the main results of this study.

An independent, non-profit organization helped prepare this summary of the study results, which included feedback from patients.

We think it is important to share the results with the participants and the public. We hope this summary helps the participants understand their important role in medical research.

The purpose of this summary is only to share information. If you need medical advice, please contact your doctor. If you participated in this study and have questions about the results, please speak with study staff.

This summary was approved by UCB Biopharma SRL on 17 December 2025. The information in this summary is current as of this date.

Overview of this study

Why was the research needed?

Researchers are looking for a different way for patients to take zilucoplan to treat their generalized myasthenia gravis.

What treatment did the participants receive?

The participants in this study gave themselves injections of zilucoplan using an auto-injector.

What were the results of the study?

The main question the researchers wanted to answer in this study was:

- **How many injections with the auto-injector were successful?**
  Overall, **99.8%** of the injections that the participants tried to give themselves with the auto-injector were successful. This was 448 out of 449 injections that the participants tried to give themselves.
  More details about the results of this study are included later in this summary.

What medical problems did the study doctors report as possibly related to study treatment?

There were 12.9% of participants (4 out of 31) who had medical problems that the study doctors reported as **possibly related** to study treatment. The most common possibly related medical problem was pain where the injection was given.

Where can I learn more about this study?

You can find more information about this study on the websites listed on the last page. If a full report of the study results is available, it can also be found on those websites.

Why was the research needed?

The researchers in this study wanted to learn about a different way for patients to take zilucoplan to treat their generalized myasthenia gravis by giving themselves injections using an auto-injector. They also wanted to learn if the participants had any medical problems during the study.

Researchers are looking for a better way to treat **myasthenia gravis**, which is a type of autoimmune disorder that makes it harder for the nerves and the muscles to communicate. Myasthenia gravis causes weakness and fatigue and requires constant and often long-term treatment to improve symptoms.

The study drug **zilucoplan** is designed to stop the immune system from attacking the body and is currently approved for treating myasthenia gravis when it is given in a pre-filled syringe. A **pre-filled syringe** is a short tube with a needle that already has the medication in it but needs someone to push the plunger down slowly to give the injection.

Some people have difficulty giving themselves an injection with a pre-filled syringe, so researchers want to know if zilucoplan can also be given in an auto-injector. An **auto-injector** is a short tube with a needle that already has the medication in it and quickly gives the injection when pressed against the skin.

Researchers want to know if participants with myasthenia gravis are able to safely give themselves a **complete dose** of zilucoplan using the auto-injector. A dose is considered "complete" when the plunger has been pushed all the way down and there is no medication left in the auto-injector.

pre-filled syringe

A device that already has the medication in it but needs someone to push the plunger

auto-injector

A device that already has the medication in it and quickly gives the injection when pressed against the skin

What was the main question studied?

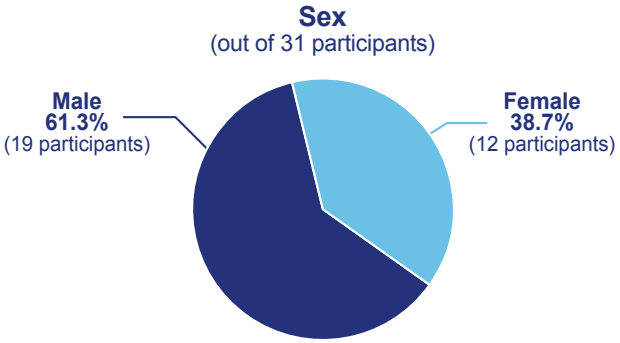
The main question the researchers wanted to answer in this study was:

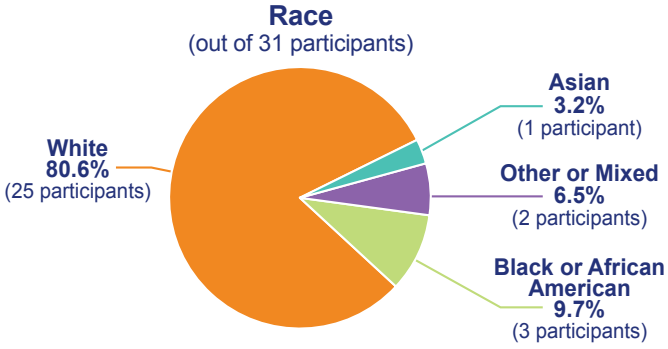
- **How many injections with the auto-injector were successful?**

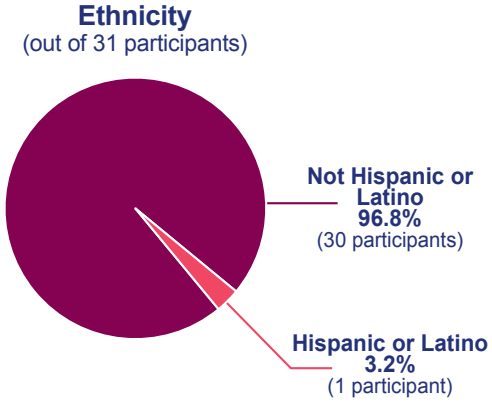
The researchers also wanted to know what medical problems happened that were possibly related to study treatment.

Who participated in the study?

There were 31 participants with generalized myasthenia gravis who participated in this study. They were 38 to 79 years old when they joined.

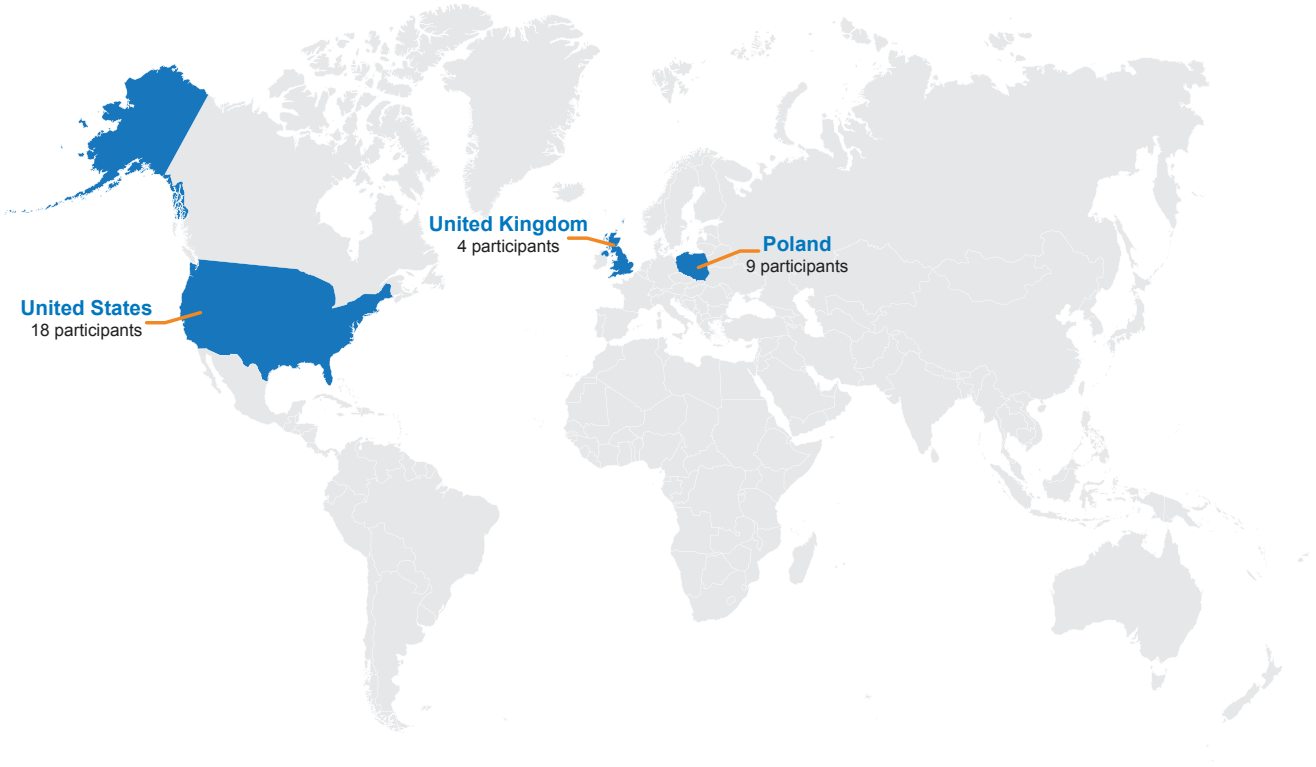






Clinical Study Results

The study included participants in 3 countries.



Zilucoplan is currently available as a prescribed medicine in a pre-filled syringe.

In this study, the researchers included participants living with generalized myasthenia gravis who:

- Were giving themselves zilucoplan as a pre-filled syringe in another study called MG0011, or were giving themselves prescribed zilucoplan as a pre-filled syringe
- Had not changed their dose of zilucoplan for at least 1 month if they were giving themselves prescribed zilucoplan

Each participant who completed the study was in the study for up to 3 weeks. The whole study lasted a little less than 6 months. The study started in August 2024 and ended in February 2025.

What treatment did the participants take?

The participants in this study gave themselves injections of zilucoplan using an auto-injector.

The participants, study doctors, study staff, and UCB staff knew what the participants were receiving.

Doses of zilucoplan are measured in milligrams (mg). The daily dose of zilucoplan that each participant received was based on their body weight:

- Participants who weighed less than 56 kg received 16.6 mg
- Participants who weighed 56 kg to 76 kg received 23.0 mg
- Participants who weighed 77 kg or more received 32.4 mg

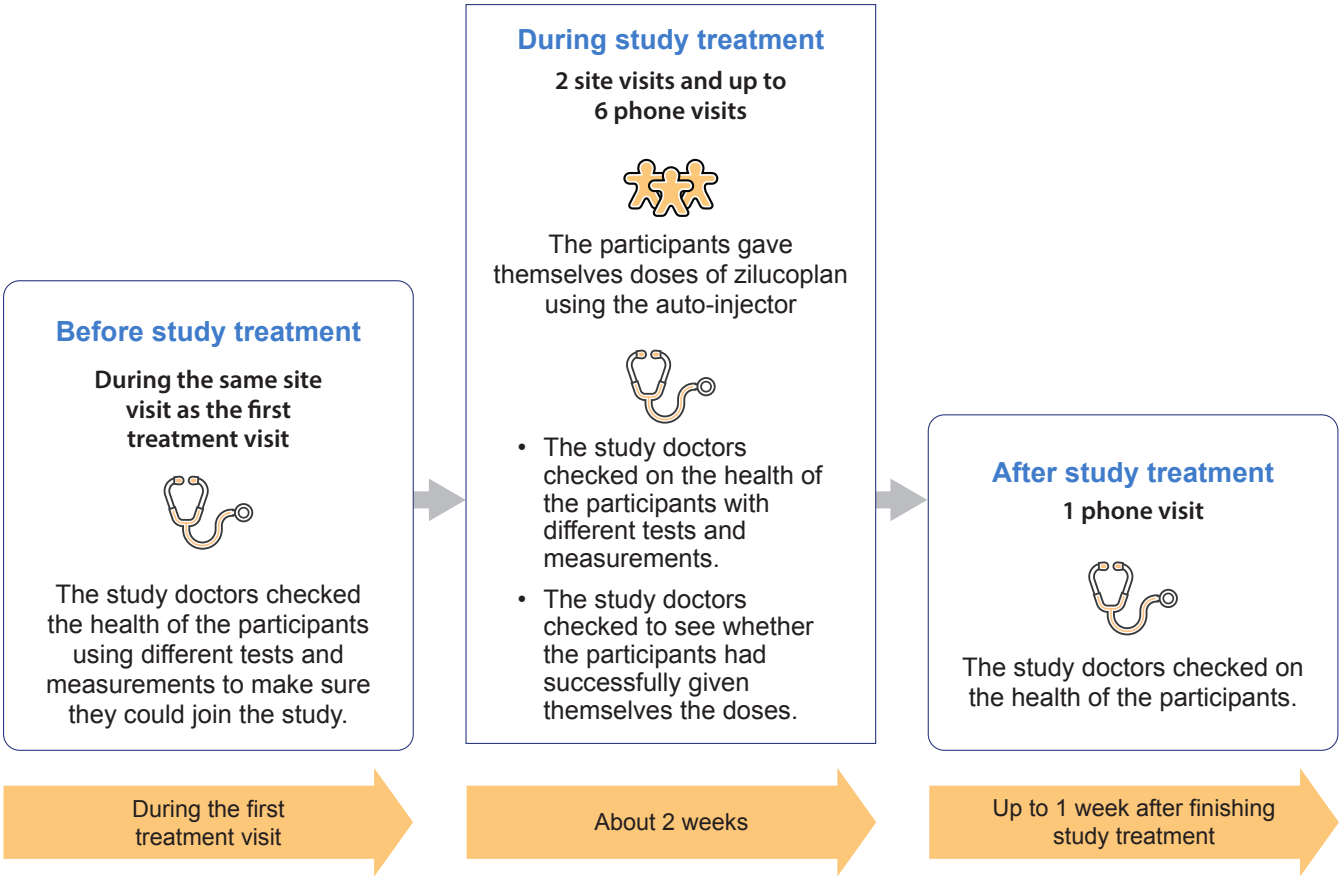
The chart below shows the treatment the researchers planned to study:

	Zilucoplan auto-injector
	31 participants
	Zilucoplan using an auto-injector
	Once a day for about 2 weeks

What happened during this study?

All the participants first learned about the study and then decided to join. This is called “informed consent”.

The chart below shows what happened in this study for each participant:



What were the results of the study?

This is a summary of the main results from this study. These are the results from all the participants combined. The individual results of each participant might be different and are not in this summary.

Deciding which treatments work best usually takes results from several studies. Other studies may provide new information or different results. Always talk to a doctor before making any treatment decisions.

How many injections with the auto-injector were successful?

The researchers found that overall, **99.8%** of the injections that the participants tried to give themselves with the auto-injector were successful. This was 448 out of 449 injections that the participants tried to give themselves.

To answer this question, the researchers kept track of each dose of zilucoplan that the participants gave themselves over about 2 weeks. The researchers also checked the used auto-injectors to make sure that the participants gave themselves a complete dose.

What medical problems did the study doctors report as possibly related to study treatment?

This section is a summary of the medical problems that the participants had during the study that the doctors reported as possibly related to study treatment. These medical problems are called “adverse reactions”.

Some participants had more than 1 adverse reaction.

This summary also includes information about **serious** adverse reactions. An adverse reaction is considered “serious” when it is life-threatening, causes lasting problems, or requires hospital care.

Other studies may or may not show that these medical problems were possibly related to study treatment. The results from several studies are often needed to decide if a treatment causes an adverse reaction. Always talk to a doctor before making any treatment decisions.

Did any adverse reactions happen during this study?

There were 12.9% of participants (4 of 31) who had an adverse reaction in this study.

Adverse reactions in this study

	Zilucoplan auto-injector (out of 31 participants)
How many participants had **serious** adverse reactions?	none
How many participants had adverse reactions?	12.9% (4 participants)
How many participants left the study due to adverse reactions?	none

What serious adverse reactions did the participants have?

There were no serious adverse reactions during this study.

What adverse reactions did the participants have?

The most common adverse reaction was pain where the injection was given.

The table below shows all the adverse reactions that happened in this study. Some participants had more than 1 adverse reaction.

Adverse reactions in this study

	Zilucoplan auto-injector (out of 31 participants)
Pain where the injection was given	9.7% (3)
A bump under the skin where the injection was given	3.2% (1)
Bleeding where the injection was given	3.2% (1)
Skin redness where the injection was given	3.2% (1)
Itchiness where the injection was given	3.2% (1)

What did the researchers learn from this study?

The results of this study have helped researchers learn more about using zilucoplan auto-injectors in people living with generalized myasthenia gravis.

In this study, the researchers found that:

- Overall, **99.8%** of the injections that the participants tried to give themselves were successful. This was 448 out of 449 injections that the participants tried to give themselves.
- There were **12.9%** of participants (4 of 31) who had an adverse reaction in this study.
- The most common adverse reaction was pain where the injection was given.

Deciding which treatments work best for patients almost always takes results from several studies. This summary shows only the main results from this one study. Other studies may provide new information or different results.

The purpose of this summary is only to share information. If you need medical advice about your own health or situation, please contact your doctor.

When this document was approved, further clinical studies with zilucoplan were planned.

Where can I learn more about this study?

You can find more information about this study at the websites listed below:

- www.clinicaltrials.gov/ct2/show/study/NCT06471361
- euclinicaltrials.eu/ctis-public/view/2023-508287-30-00

If you have questions about this study, UCB contact information is available at https://www.ucb.com/UCBCares.

Study Information

Protocol Number: DV0013

National Clinical Trial Number: NCT06471361

EU CT Number: 2023-508287-30-00

WHO ICTRP: U1111-1302-4369

Study Sponsor: UCB Biopharma SRL sponsored this study. They are referred to as UCB in this summary.

Full Study Title: A Multicenter, Open-Label, Outpatient Study to Evaluate the Safe And Effective Use of a Zilucoplan Auto-Injector Combination Product for Subcutaneous Self-Administration by Study Participants With Generalized Myasthenia Gravis

Thank you

Participants in clinical studies belong to a large community of people who take part in clinical research around the world. They help researchers answer important health questions and find medical treatments for patients.

CISCRP REVIEW PANEL

This summary was last updated on 17 December 2025.
The final clinical study report is dated 25 July 2024.