Clinical Study Results

Inspired by patients. Driven by science.

Study Sponsor: UCB Biopharma SRL

Treatment Studied: Zilucoplan

Protocol Number: MG0017

Short Study Title: A study to learn about the safety of zilucoplan in people with generalized myasthenia gravis who were previously treated with complement 5 inhibitors through an IV infusion

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 September 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 to treat generalized myasthenia gravis. Researchers do clinical studies to find out how treatments work and how safe they are.

What treatment did the participants receive?

The participants in this study received zilucoplan. All the participants had previously been receiving a type of treatment called a complement 5 inhibitor.

This study had 2 parts: a main treatment period and an optional extension treatment period. The **main treatment period** lasted 12 weeks.

More details about zilucoplan and the treatment periods are included later in this summary.

What were the results of this study?

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

- **What medical problems did the participants have during the main treatment period of the study?**
  - There were **73.1%** of participants (19 out of 26) who had a medical problem during the **main treatment period** with zilucoplan.
  - The **most common** medical problem was a temporary increase in the levels of a protein called **amylase** in the blood, which may be a sign of pancreas injury.

More details about the results of this study are included later in this summary.

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

There were **38.5%** of participants (10 out of 26) who had medical problems **during the whole study** that the study doctors reported as **possibly related** to study treatment.

The **most common** possibly related medical problem was a temporary increase in the levels of a protein called **lipase** in the blood, which may be a sign of pancreas injury.

The whole study included the main treatment period and the optional extension treatment period.

Where can I learn more about this study?

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

Why was the research needed?

Researchers do clinical studies to get information about how well the treatment works and about how safe it is.

The **immune system** is the body's natural defense system. It fights diseases, infections, and anything it does not recognize as a normal part of the body. There are many proteins that help the immune system protect the body. But, in people with diseases of the immune system, these proteins can cause the immune system to attack the body's own healthy cells. These types of diseases of the immune system are called **autoimmune diseases**.

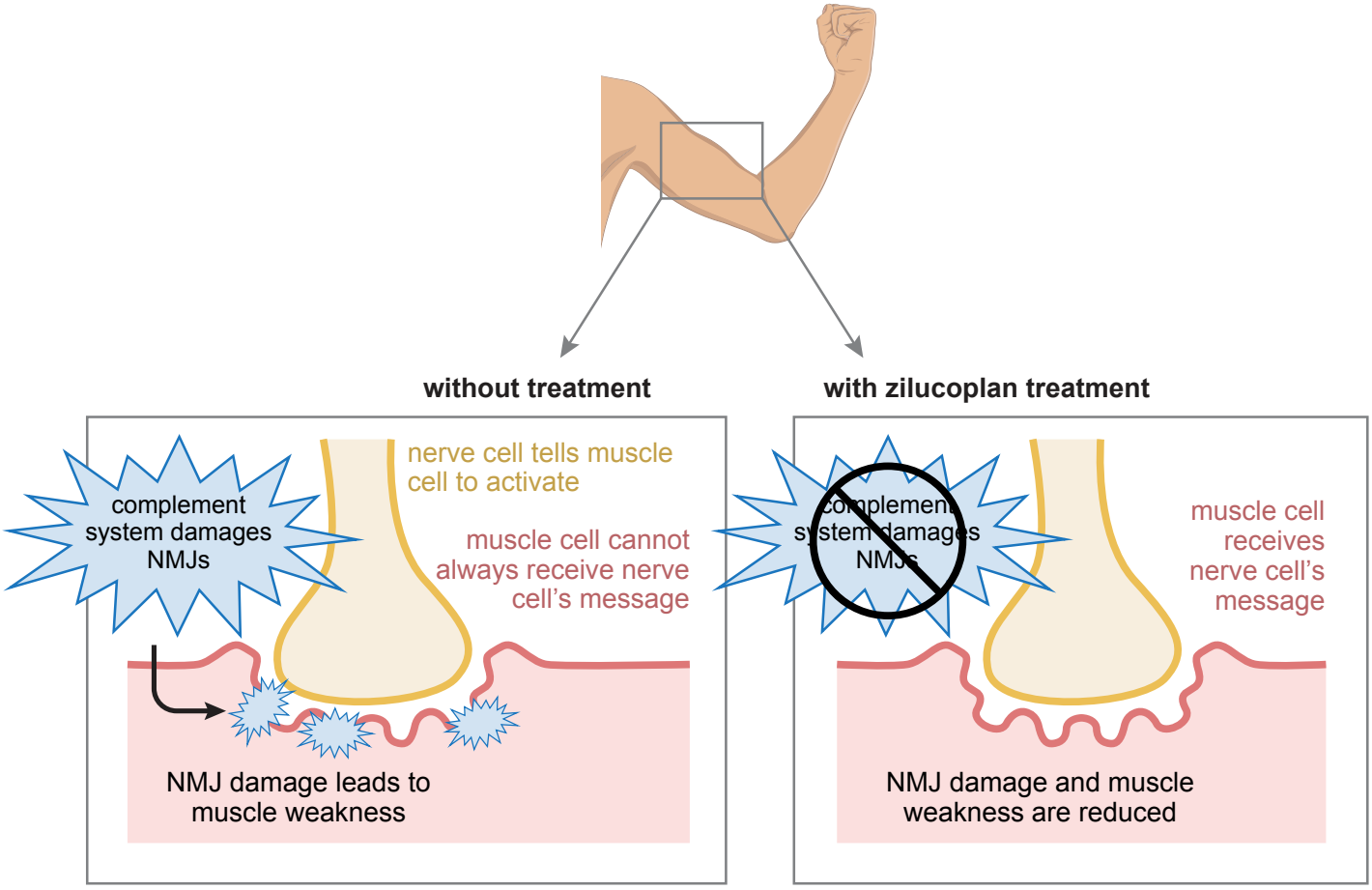
Myasthenia gravis is a rare autoimmune disease that makes it harder for the nerves and the muscles to communicate. In people with myasthenia gravis, the immune system attacks the places where nerve cells connect to muscle cells. These are called **neuromuscular junctions** (**NMJs**). When NMJs are damaged, this can lead to extreme muscle weakness.

Generalized myasthenia gravis is when muscles throughout the body are affected. This can have an impact on several body functions, including seeing objects clearly, speech, and swallowing. It can also affect the limbs. These symptoms make it difficult for people to do everyday activities. Generalized myasthenia gravis is a condition that often requires long-term treatment to improve symptoms.

The participants in this study had generalized myasthenia gravis and had previously been receiving a type of treatment called a complement 5 inhibitor through a needle directly into a vein over a period of time (IV infusion). **Complement 5 inhibitors** are designed to stop part of the immune system called the **complement system** from attacking the NMJs in the body.

The study drug **zilucoplan** is a type of complement 5 inhibitor that can be received as an injection just under the skin. These injections are often more convenient for people than an IV infusion, so zilucoplan could be a more appealing treatment option than other complement 5 inhibitors.

The researchers in this study wanted to learn about the safety of zilucoplan in participants living with generalized myasthenia gravis who were previously receiving a complement 5 inhibitor through an IV infusion.



What was the main question studied?

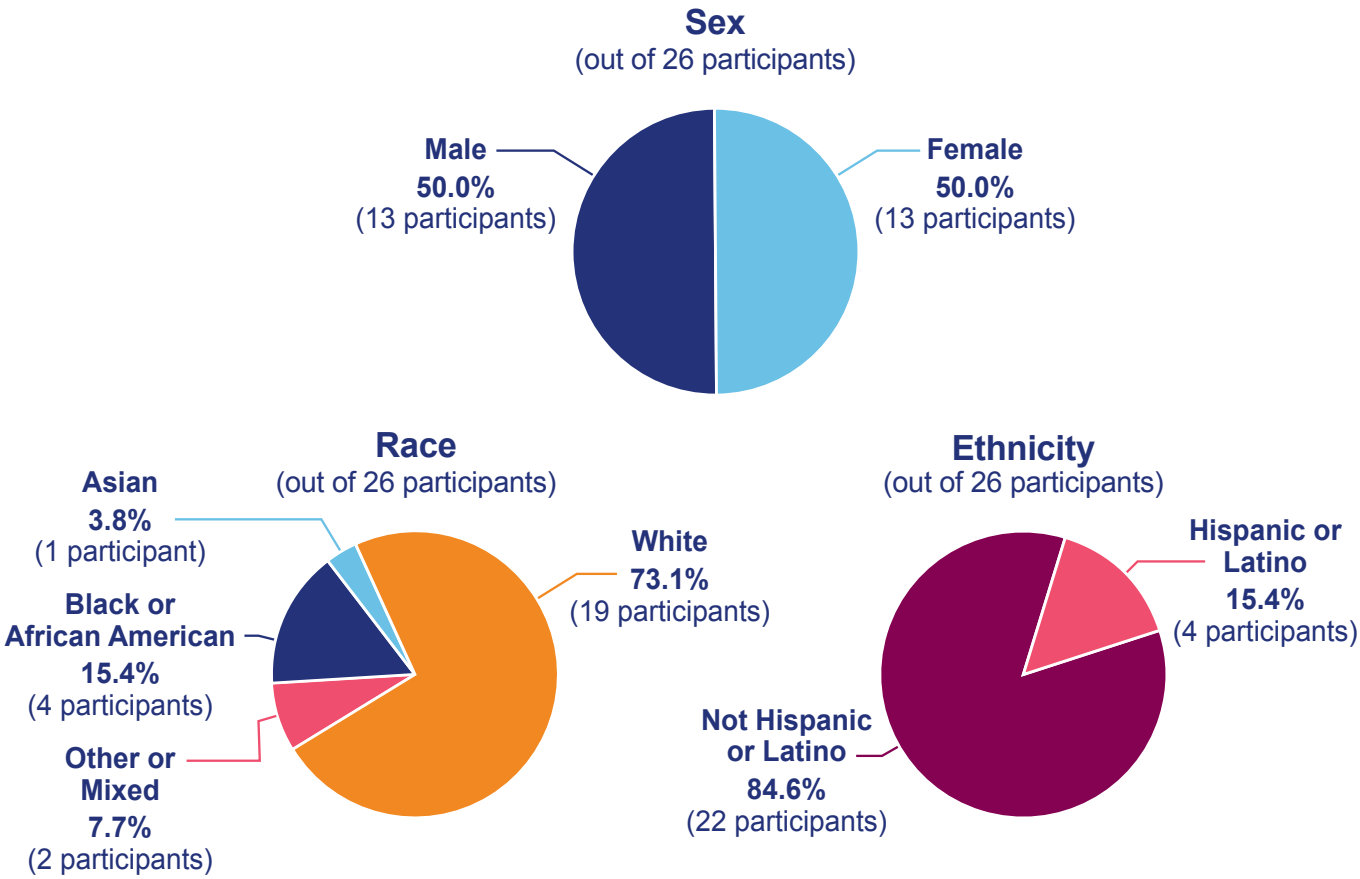
The main question the researchers wanted to answer in this study was:

- **What medical problems did the participants have during the main treatment period of the study?**

The researchers also wanted to know what medical problems happened that were possibly related to study treatment during the whole study. This included the main treatment period and the optional extension treatment period.

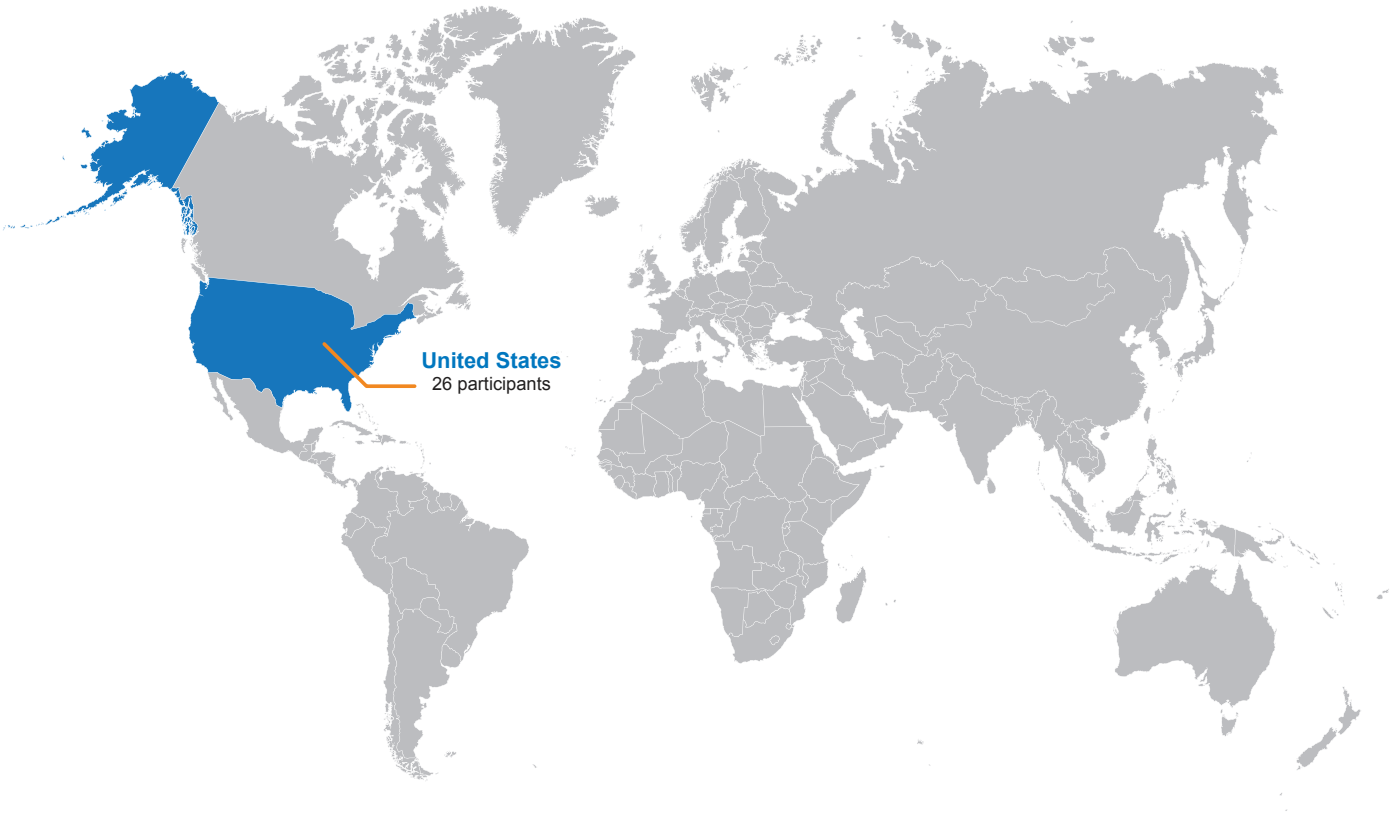
Who participated in the study?

There were 26 participants with generalized myasthenia gravis who participated in this study. They were 19 to 82 years old when they joined.



Clinical Study Results

The study included participants in the United States.



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

- Had been receiving a complement 5 inhibitor through an IV infusion for at least 3 or 4 months before joining the study
- Had stable generalized myasthenia gravis
- Had antibodies that attacked the acetylcholine receptors on their muscle cells

Acetylcholine receptors help certain nerve cells communicate with muscle cells.

All participants had to be vaccinated against meningococcal infection at the start of the study. This is a type of bacterial infection that can lead to meningitis, which is an infection of the lining of the brain and spinal cord. If a participant was not vaccinated, they had to receive a vaccination before they could receive zilucoplan.

Each participant received zilucoplan until it was approved and made available for use in the United States, or until they left the study.

The whole study lasted 2 years. The study started in October 2022 and ended in October 2024.

What treatment did the participants receive?

The participants in this study received zilucoplan. Doses of zilucoplan were measured in milligrams per kilogram of body weight (mg/kg).

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

This study had 2 parts: the main treatment period and the optional extension treatment period. The **main treatment period** lasted 12 weeks. The **optional extension treatment period** lasted until zilucoplan was approved and made available for use in the United States or until the participants left the study for a different reason.

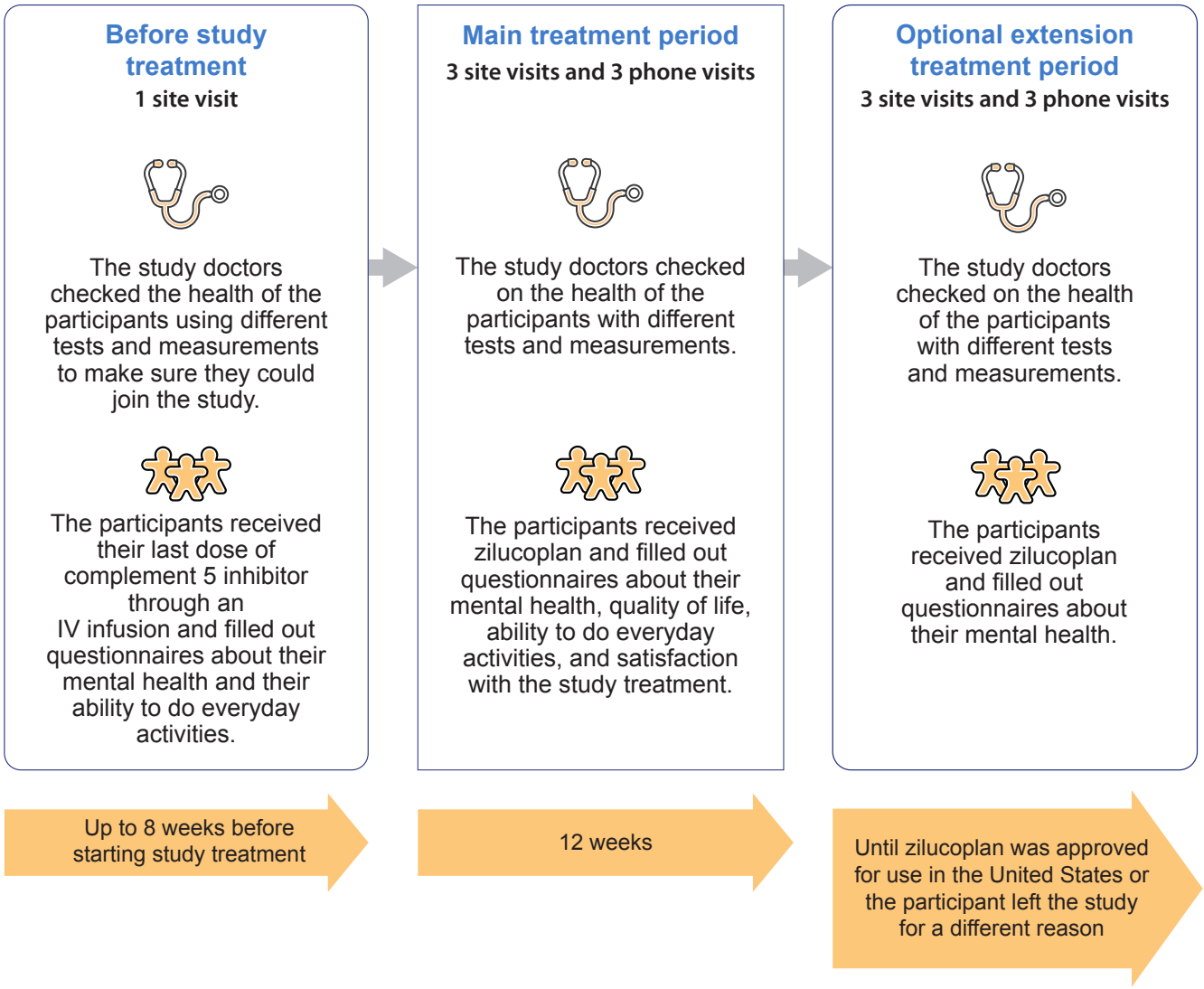
The chart below shows the treatment the researchers studied:

	26 participants
	0.3 mg/kg of zilucoplan as an injection just under the skin
	Once a day for both parts of the study

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:



Participants who stopped receiving zilucoplan early had 1 last site visit about 40 days after stopping zilucoplan where the study doctors checked on their health.

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.

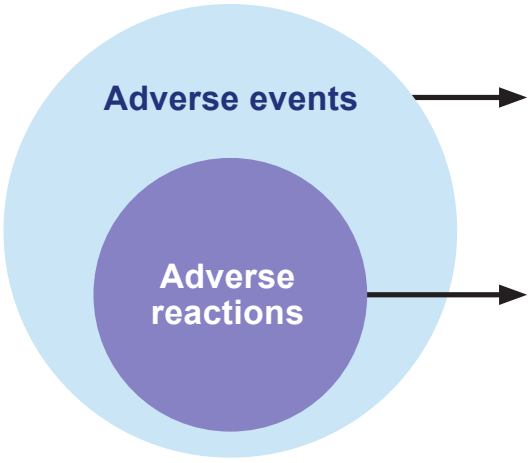
Deciding which treatments work best usually takes results from several studies. Other studies may provide new information or different results. Always talk to a doctor before making any treatment decisions.

What medical problems did the participants have during the main treatment period of the study?

In this summary, there is information about 2 different types of medical problems that the participants had during the study. An **adverse event** is **any** medical problem that a participant has during a study. Doctors keep track of all adverse events that happen in studies, whether or not these may be related to study treatment.

An **adverse reaction** is different from an adverse event because it is reported by the doctor as **possibly related** to study treatment. An adverse event or adverse reaction is considered “serious” when it is life-threatening, causes lasting problems, or requires hospital care.

Adverse reactions are adverse events that the study doctors reported as possibly related to study treatment



Adverse events are all of the medical problems the participants had during this study.

Adverse reactions are medical problems the participants had during this study that the study doctors reported as possibly related to study treatment.

The information below is a summary of the **adverse events** that happened in the **main treatment period** of this study. The main treatment period lasted 12 weeks. The results from the optional extension treatment period helped the researchers learn more about zilucoplan but are not included in this part of the summary.

There were 73.1% of participants (19 of 26) who had an adverse event in the main treatment period of this study.

Adverse events in the main treatment period of this study

	Zilucoplan (out of 26 participants)
How many participants had **serious** adverse events?	**3.8%** (1 participant) 
How many participants had adverse events?	**73.1%** (19 participants) 
How many participants stopped receiving study treatment due to adverse events?	**7.7%** (2 participants) 

The **serious** adverse events in the **main treatment period** of this study were:

- Inflammation or infection in the small pouches of the large intestine (Diverticulitis)
- Infection of the kidneys, usually caused by bacteria (Pyelonephritis)

Both serious adverse events happened in the same participant.

The most common adverse events in the **main treatment period** of this study were:

- A temporary increase in the levels of a protein called amylase in the blood, which may be a sign of pancreas injury
- Inflammation of the sinuses (Sinusitis)
- Pain where the injection was given
- Pain
- Diarrhea
- Nausea
- A temporary increase in the levels of a protein called lipase in the blood, which may be a sign of pancreas injury

There were 2 participants who stopped receiving study treatment during the main treatment period due to adverse events. The adverse events that led to these participants leaving the main treatment period of this study included:

- Feeling tired (Fatigue)
- Changed skin color where the injection was given
- Pain where the injection was given
- Pain
- Infection with a common virus called Epstein-Barr virus, which can cause mononucleosis, also known as “mono”
- Anxiety

These results are from the **main treatment period** only.

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

This section is a summary of the medical problems that the participants had during the **whole study** that the doctors reported as **possibly related** to study treatment. These medical problems are called “**adverse reactions**”. The results in this section include the adverse reactions that happened in either part of this study.

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.

Some of the adverse reactions listed below may also be listed in the adverse events section earlier in this summary.

Did any adverse reactions happen during this study?

There were 38.5% of participants (10 of 26) who had an adverse reaction in either part of this study.

Adverse reactions in either part of this study

	Zilucoplan (out of 26 participants)
How many participants had **serious** adverse reactions?	**none** 
How many participants had adverse reactions?	**38.5%** (10 participants) 
How many participants stopped receiving study treatment due to adverse reactions?	**7.7%** (2 participants) 

What adverse reactions did the participants have?

The most common adverse reaction was a temporary increase in the levels of a protein called lipase in the blood, which may be a sign of pancreas injury.

The table below shows the adverse reactions that happened in 2 or more participants in either part of this study. There were other adverse reactions, but those happened in only 1 participant each.

Adverse reactions in 2 or more participants in either part of this study

	Zilucoplan (out of 26 participants)
A temporary increase in the levels of a protein called lipase in the blood, which may be a sign of pancreas injury	11.5% (3)
Bruising where the injection was given	7.7% (2)
Pain where the injection was given	7.7% (2)
Itchiness where the injection was given	7.7% (2)
A temporary increase in the levels of a protein called amylase in the blood, which may be a sign of pancreas injury	7.7% (2)

What did the researchers learn from this study?

The results of this study have helped researchers learn more about using zilucoplan after previous treatment with a different complement 5 inhibitor in people living with generalized myasthenia gravis.

In this study, the researchers found that:

- 73.1% of participants (19 out of 26) had medical problems during the 12-week main treatment period. The most common medical problem was a temporary increase in the levels of a protein called amylase in the blood, which may be a sign of pancreas injury.
- 38.5% of participants (10 out of 26) had medical problems during either part of the study that were possibly related to study treatment.

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.

At the time this document was approved, further clinical studies in generalized myasthenia gravis with zilucoplan were ongoing.

Where can I learn more about this study?

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

- www.clinicaltrials.gov/ct2/show/study/NCT05514873

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

Study Information

Protocol Number: MG0017

National Clinical Trial Number: NCT05514873

Study Sponsor: UCB Biopharma SRL sponsored this study. It is referred to as UCB in this summary.

Full Study Title: A Phase 3b, Multicenter, Open-Label, Single-Arm Study to Evaluate the Safety, Tolerability, and Efficacy of Zilucoplan in Participants With Generalized Myasthenia Gravis Switching From Intravenous Complement Component 5 Inhibitors to Subcutaneous Zilucoplan

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 September 2025.
The final clinical study report is dated 11 March 2025.