Clinical Study Results

Inspired by patients. Driven by science.

Study Sponsor: UCB Biopharma SRL

Drug Studied: Rozanolixizumab

Protocol Number: MG0007

Short Study Title: A study to learn about the long-term safety of rozanolixizumab and how well it works in people with generalized myasthenia gravis

Thank you

UCB thanks all the participants of this study. All the participants helped the researchers learn more about using rozanolixizumab 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 23 January 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. Before a drug is available for all patients, researchers do clinical studies to find out how the drug works and how safe it is.

What treatment did the participants receive?

The participants in this study received rozanolixizumab.

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 this study?**

  90.4% of the participants (142 out of 157) had medical problems during the study. The most common medical problem was headache.

The researchers also wanted to know:

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

  There were 56.1% of participants (88 out of 157) who had medical problems that the study doctors reported as **possibly being related** to the study drug. The most common possibly related medical problem was headache.

- **Did rozanolixizumab improve the participants' generalized myasthenia gravis symptoms?**

  Overall, the researchers found that the participants had improvements in their generalized myasthenia gravis symptoms over time.

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

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 also can be found on those websites.

Why was the research needed?

Before a treatment is available to the public, researchers do clinical studies to get information about how well the treatment works and about how safe it is.

All of the participants in this study were also in a previous study called MG0003 that looked at how well rozanolixizumab works and how safe it is in a large number of participants living with generalized myasthenia gravis. The researchers in this study wanted to learn more about the safety of rozanolixizumab and how well it works over a long period of time.

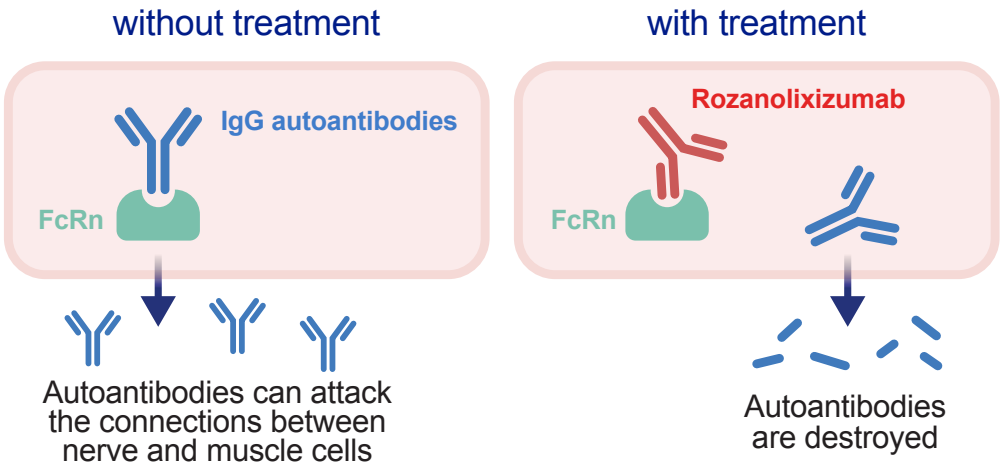
The **immune system** is the body's natural defense system. It fights diseases, infections, and anything it does not recognize as a normal part of the body. But, in people with diseases of the immune system, the immune system attacks the body's own healthy cells. These types of diseases of the immune system are called **autoimmune diseases**.

Generalized myasthenia gravis is a rare autoimmune disease that can lead to extreme muscle weakness throughout the body. This can affect activities or body functions that many people take for granted, including seeing objects clearly, speech, and swallowing. It can also affect the limbs, making it difficult for people to do everyday activities, including working or studying. It is a long-term condition that requires constant and often long-term treatment to improve symptoms.

In people living with generalized myasthenia gravis, proteins in the immune system called **IgG autoantibodies** attack the connections between nerve cells and muscle cells, causing muscle weakness. IgG autoantibodies can also attach to a protein called **FcRn**, which protects them from breaking down and allows them to continue to attack the body for a longer time.

The study drug **rozanolixizumab** is designed to stop antibodies, including IgG autoantibodies, from attaching to FcRn. When these antibodies cannot attach to FcRn, they are broken down by the body more quickly. While lower levels of antibodies in the body may increase the risk of infection, researchers think that lower levels of IgG autoantibodies may help to improve the symptoms of people with generalized myasthenia gravis.

How rozanolixizumab is designed to work



What were the main questions studied?

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

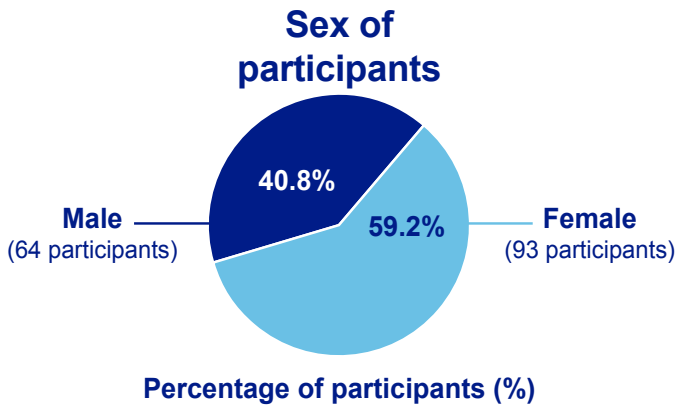
- What medical problems did the participants have during this study?

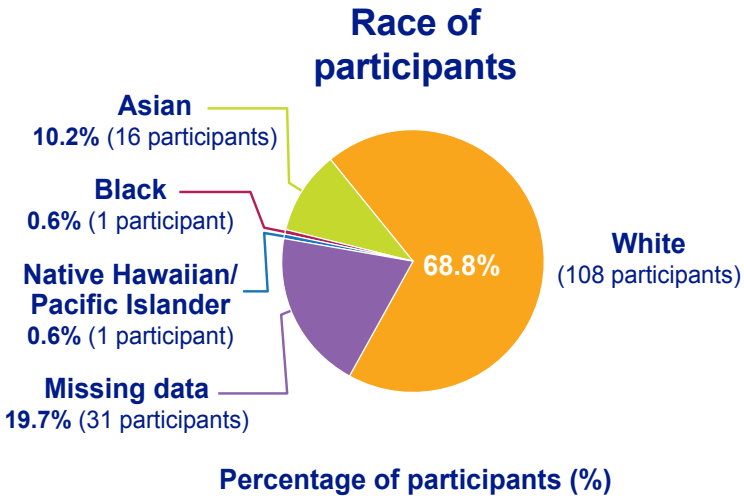
The researchers also wanted to know:

- What medical problems did the study doctors report as possibly related to study treatment?
- Did rozanolixizumab improve the participants' generalized myasthenia gravis symptoms?

Who participated in the study?

There were 157 participants with generalized myasthenia gravis who received study treatment. They were 18 to 85 years old when they joined.





Some of the data about race and ethnicity is listed as “missing” because certain countries do not allow this information to be collected from study participants.

Clinical Study Results

The study included participants in 14 countries.

This study included participants living with generalized myasthenia gravis who were in the MG0003 study and had met any of the following:

- Completed MG0003.
- Did not complete MG0003, but went to at least 6 visits in another related study called MG0004.
- Needed rescue treatment for their generalized myasthenia gravis after they finished receiving rozanolixizumab in MG0003.

To be able to join MG0003, participants must have had:

- Mild to severe muscle weakness.
- An MG-ADL score of at least 3 and a QMG score of at least 11. MG-ADL and QMG are scoring systems that doctors use to determine how severe a person's generalized myasthenia gravis is.
- Tests that showed they had autoantibodies against the proteins AChR or MuSK (or both) in their blood.
- Been considered for certain different treatments for their generalized myasthenia gravis by their doctor before joining this study.

Each participant who completed the study was in the study for up to a little less than 3 years. The study started in February 2021 and ended in January 2024.

What treatment did the participants receive?

The participants in this study received rozanolixizumab through a needle just under the skin. Doses of rozanolixizumab were measured in milligrams per kilogram of body weight, also called mg/kg.

Participants were split into groups based on their weight. Participants in each weight group were assigned to receive about 7 mg/kg or about 10 mg/kg of rozanolixizumab. For participants who were previously in the MG0004 study, the dose was based on what they were receiving in MG0004. For participants who were previously only in the MG0003 study, the dose was randomly assigned. During this study, the study doctors could switch each participant's dose level if they thought it would help.

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Participants in this study received rozanolixizumab in cycles. In each cycle, participants received rozanolixizumab for 6 weeks and then received no treatment for at least 4 weeks afterwards. Then, participants could begin a new cycle of treatment if the study doctors thought it would help them.

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

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

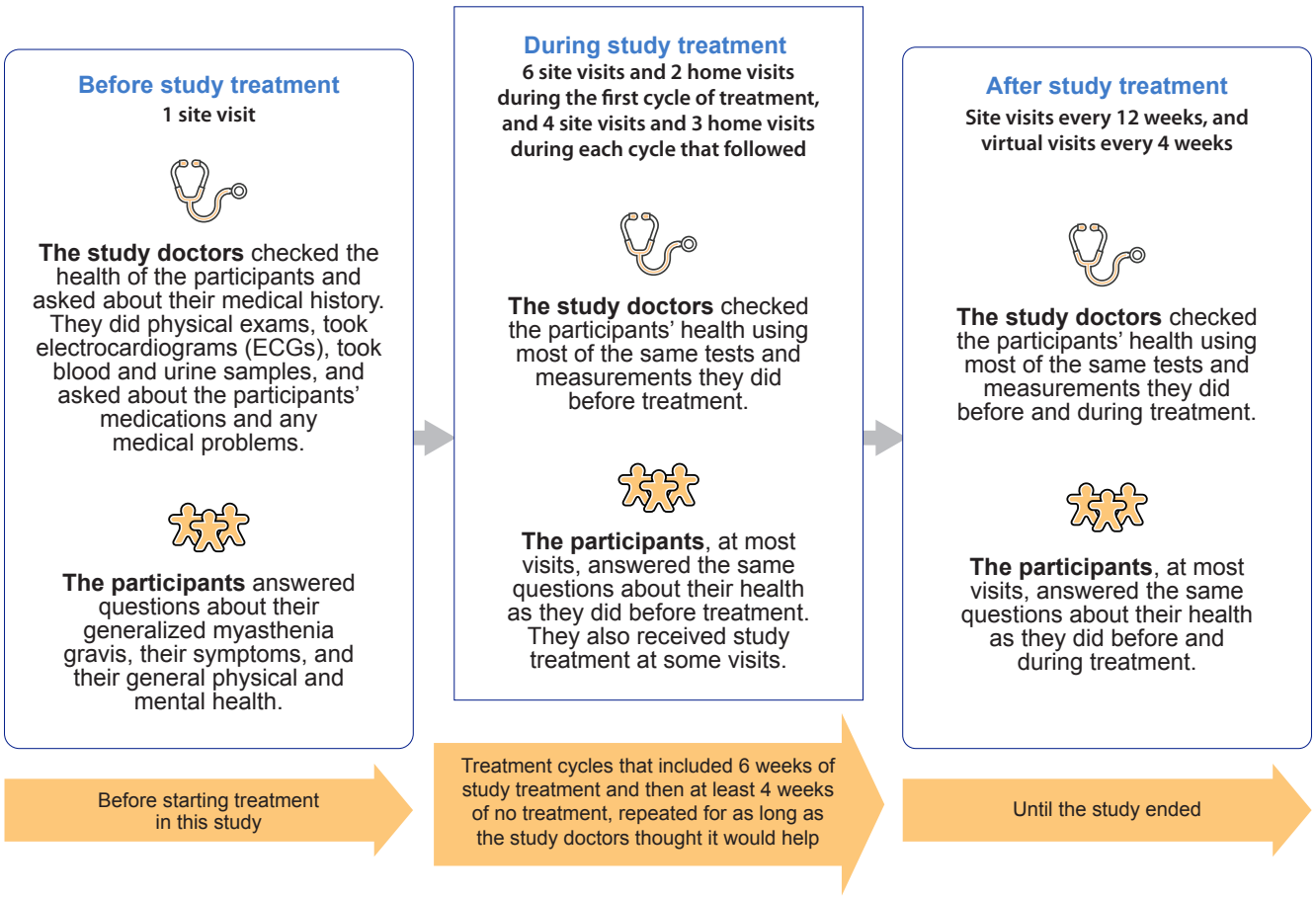
	157 participants
	7 mg/kg or 10 mg/kg of rozanolixizumab through a needle just under the skin
	Once a week for 6 weeks, then no treatment for at least 4 weeks This could be repeated if study doctors thought it would help

The participants also took most of their regular treatments for generalized myasthenia gravis.

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?

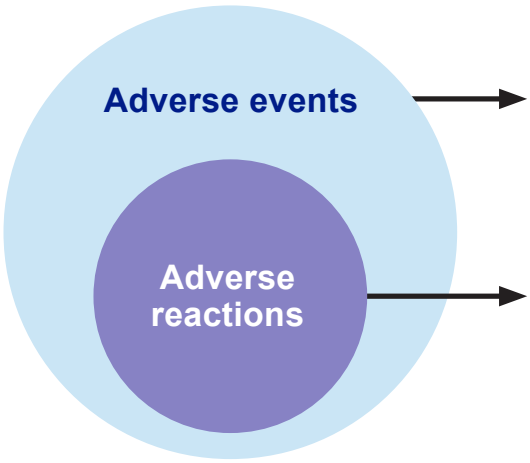
This is a summary of the main results from this study. These are the results from all the participants combined. The individual results of each participant might be different and are not in this summary.

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.

What medical problems did the participants have during this 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 the study treatment. An **adverse reaction** is different from an adverse event because it is reported by the doctor as **possibly related** to the 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 think may be 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 this study.

There were 90.4% of participants (142 of 157) who had an adverse event in this study.

The doctors may have switched the dose of rozanolixizumab each participant received if they thought it was best. The adverse events in this section are shown based on which dose of rozanolixizumab the participants received most recently before the adverse event. This means that some participants are in both groups. The last column in each table shows the total adverse events for all participants.

Adverse events in this study

	7 mg/kg of rozanolixizumab (out of 102 participants)	**10 mg/kg of rozanolixizumab** (out of 102 participants)	**Any dose of rozanolixizumab** (out of 157 participants)
How many participants had **serious** adverse events?	15.7% (16 participants)	30.4% (31 participants)	28.7% (45 participants)
How many participants had adverse events?	78.4% (80 participants)	94.1% (96 participants)	90.4% (142 participants)
How many participants stopped receiving study treatment due to adverse events?	9.8% (10 participants)	16.7% (17 participants)	17.2% (27 participants)

The most common **serious** adverse events were:

- Worsening myasthenia gravis
- Myasthenia gravis crisis, which may have included difficulty breathing
- COVID-19 infection

The most common adverse events were:

- Headache
- Diarrhea
- COVID-19 infection

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.

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

The participants in this study may have switched the dose of rozanolixizumab they were receiving if the study doctors thought it was best. The adverse reactions are shown based on which dose of rozanolixizumab the participants received most recently before the adverse reaction. This means that some participants are in both groups. This summary also includes the total adverse reactions for all participants.

Did any adverse reactions happen during this study?

There were 56.1% of participants (88 of 157) who had an adverse reaction in this study. Information about the adverse reactions for participants based on their most recent dose of rozanolixizumab is shown below.

Adverse reactions in this study

	7 mg/kg of rozanolixizumab (out of 102 participants)	**10 mg/kg of rozanolixizumab** (out of 102 participants)	**Any dose of rozanolixizumab** (out of 157 participants)
How many participants had **serious** adverse reactions?	2.9% (3 participants)	1.0% (1 participant)	2.5% (4 participants)
How many participants had adverse reactions?	42.2% (43 participants)	61.8% (63 participants)	56.1% (88 participants)
How many participants left the study due to adverse reactions?	2.9% (3 participants)	2.0% (2 participants)	3.2% (5 participants)

What serious adverse reactions did the participants have?

The table below shows the serious adverse reactions that happened during the study. Some of the participants had more than 1 serious adverse reaction.

Serious adverse reactions			
Serious adverse reaction	**7 mg/kg of rozanolixizumab** (out of 102 participants)	**10 mg/kg of rozanolixizumab** (out of 102 participants)	**Any dose of rozanolixizumab** (out of 157 participants)
Fever	1.0% (1)	none	1.0% (1)
Inflammation or infection in the small pouches of the large intestine (Diverticulitis)	1.0% (1)	none	1.0% (1)
Inflammation of the sinuses due to an infection with a fungus called Aspergillus (Sinusitis aspergillus)	1.0% (1)	none	1.0% (1)
Inflammation of the skin due to a disease called lupus (Subacute cutaneous lupus erythematosus)	1.0% (1)	none	1.0% (1)
Infection in the protective covering of the brain and spinal cord that is not caused by bacteria (Meningitis aseptic)	none	1.0% (1)	1.0% (1)

None of the participants died due to serious adverse reactions.

What adverse reactions did the participants have?

The most common adverse reaction was headache.

The table below shows the adverse reactions that happened in 5.0% or more of participants overall. There were other adverse reactions, but those happened in fewer participants.

Adverse reactions in 5.0% or more of participants

Adverse reaction	7 mg/kg of rozanolixizumab (out of 102 participants)	10 mg/kg of rozanolixizumab (out of 102 participants)	Any dose of rozanolixizumab (out of 157 participants)
Headache	22.5% (23)	34.3% (35)	31.8% (50)
Diarrhea	11.8% (12)	17.6% (18)	16.6% (26)
Decreased levels of IgG in the blood	5.9% (6)	13.7% (14)	12.7% (20)
Fever	4.9% (5)	11.8% (12)	10.2% (16)
Nausea	5.9% (6)	6.9% (7)	7.0% (11)
Stomach pain	4.9% (5)	2.9% (3)	5.1% (8)

The researchers expected participants to have decreased levels of IgG in the blood because this is how rozanolixizumab is designed to work in the body.

Did rozanolixizumab improve the participants' generalized myasthenia gravis symptoms?

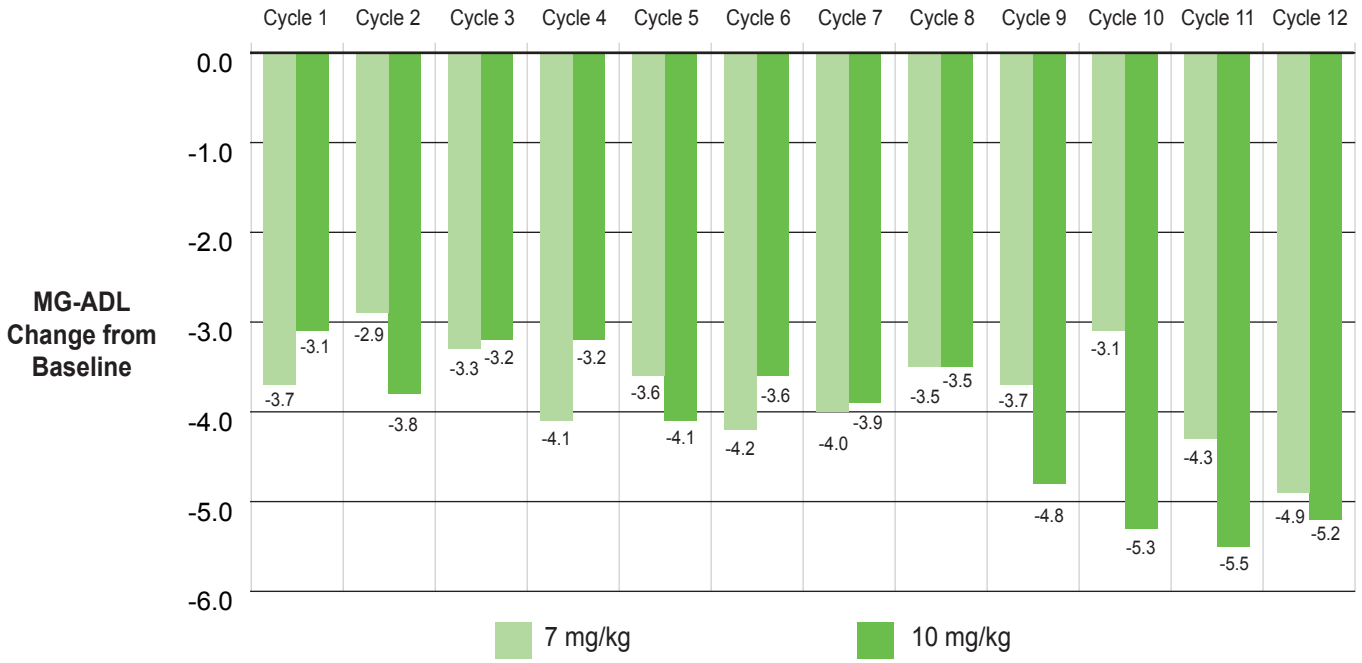
Overall, throughout the study, the researchers found that the participants had improvements in their generalized myasthenia gravis symptoms.

To test this, the participants answered a questionnaire about their generalized myasthenia gravis symptoms and their ability to do their normal daily activities. This questionnaire is called **Myasthenia Gravis Activities of Daily Living** (**MG-ADL**).

The participants' answers on the questionnaire were given a score. A higher score meant that a person's generalized myasthenia gravis was more severe. The researchers compared each participant's score from before they received study treatment to their score at different times after they had started receiving study treatment. If a participant's scores had **decreased** over time, this meant that their generalized myasthenia gravis symptoms had **improved**.

The amount that the participants' overall MG-ADL scores decreased 1 week after finishing treatment with rozanolixizumab in each cycle is shown below.

Changes in MG-ADL scores at Day 43 in each cycle of rozanolixizumab compared to their score before treatment



The researchers found that overall, the participants' MG-ADL scores decreased after treatment with rozanolixizumab, and their decrease in scores continued with further cycles of treatment. This means that the participants' myasthenia gravis symptoms improved and these improvements continued with repeated rozanolixizumab treatment.

What did the researchers learn from this study?

The results of this study have helped researchers learn more about using rozanolixizumab as needed over a long period of time in people living with generalized myasthenia gravis. In this study, the researchers found that:

- 90.4% of participants had a medical problem during this study that may or may not have been related to study treatment.
- 56.1% of participants had a medical problem that was possibly related to study treatment (adverse reaction). The most common adverse reaction was headache.
- 2.5% of participants had a **serious** adverse reaction.
- Participants had improvements in their generalized myasthenia gravis symptoms in each treatment cycle.

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 rozanolixizumab 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/NCT04650854
- www.clinicaltrialsregister.eu/ctr-search/search?query=2020-003230-20

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

Study Information

Protocol Number: MG0007

National Clinical Trial Number: NCT04650854

EudraCT Number: 2020-003230-20

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

Full Study Title: An Open-Label Extension Study to Evaluate Rozanolixizumab in 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.

This summary was last updated on 23 January 2025.
The final clinical study report is dated 12 June 2024.