Clinical Study Results

Inspired by patients. Driven by science.

Study Sponsor: UCB Biopharma SRL

Drug Studied: Rozanolixizumab

Protocol Number: MG0003

Study Purpose: A study to learn if rozanolixizumab works in people with generalized myasthenia gravis and about its safety

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**. Rozanolixizumab is also called UCB7665.

This study is also called the **MyCarinG** study. 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.

Overview of this study

Why was the research needed?

Researchers are looking for a different way to treat generalized myasthenia gravis. Before a treatment is available for all patients, researchers do clinical studies to find out if the treatment works and how safe it is.

What treatments did the participants receive?

The participants in this study received rozanolixizumab or a placebo. The placebo was given in the same way as rozanolixizumab but did not have any rozanolixizumab in it.

What were the results of the study?

The main questions the researchers wanted to answer in this study were:

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

  Yes. The researchers found that the participants who received rozanolixizumab had improvements in their generalized myasthenia gravis symptoms compared to the participants who received the placebo.

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

  There were **46.5%** of participants who had medical problems that the study doctors reported as possibly being related to the study treatment. This was 93 out of 200 participants.

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 can also be found on those websites.

Why was the research needed?

Before a treatment is available to all patients, 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 can lead to extreme muscle weakness. Myasthenia gravis can be ocular or generalized. It is called **ocular myasthenia gravis** when only the muscles that move the eyes and eyelids are affected. It is called **generalized myasthenia gravis** when muscles throughout the body, which may include the eyes, are affected. 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. The participants in this study had generalized myasthenia gravis.

Our body uses chemical transmitters and specific proteins (receptors) for passing messages between nerves and muscles. In people living with generalized myasthenia gravis, proteins in the immune system called **IgG autoantibodies** attack these receptors. Most people with myasthenia gravis have autoantibodies to acetylcholine receptors (**AChR**), and a smaller number have autoantibodies to muscle-specific kinase (**MuSK**).

IgG autoantibodies can attach to another protein called **FcRn**, which allows them to continue to attack the body's own receptors. The study drug **rozanolixizumab** is designed to stop IgG autoantibodies from attaching to FcRn. When IgG autoantibodies are stopped from attaching to FcRn, they are broken down by the body more quickly. Researchers think this will reduce the levels of IgG autoantibodies in the body, which may help to improve the symptoms experienced by people with generalized myasthenia gravis.

The researchers in this study wanted to learn if rozanolixizumab worked in a large number of participants living with generalized myasthenia gravis. They also wanted to learn if the participants had any medical problems during the study.

What were the main questions studied?

The main questions the researchers wanted to answer in this study were:

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

Who participated in the study?

There were 79 males and 121 females with generalized myasthenia gravis who participated in this study. They were 18 to 89 years old when they joined.

The study included participants in 15 countries:

Country	Number of participants
Canada	19
Czech Republic	3
Denmark	5
France	18
Georgia	13
Germany	9
Hungary	1
Italy	13

Country	Number of participants
Japan	13
Poland	27
Russia	14
Serbia	9
Spain	8
Taiwan	7
The United States of America	41

In this study, the researchers planned to include participants living with generalized myasthenia gravis who 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 2 scoring systems that doctors use to determine how severe a person's generalized myasthenia gravis is.
- Had tests that showed they had AChR or MuSK autoantibodies (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 was in the study for up to about 18 weeks, but the whole study lasted for nearly 2 years and 5 months. The study started in June 2019 and ended in October 2021.

What treatments did the participants receive?

The participants in this study received rozanolixizumab or a placebo. They received these over a period of time through a needle just under the skin that was attached to an infusion line, which is called a **subcutaneous infusion**. The placebo was given in the same way as rozanolixizumab but did not have any rozanolixizumab in it. The researchers used the placebo infusions to help make sure the effects they found in the study were actually caused by rozanolixizumab.

The participants could also continue to take some standard of care treatments for generalized myasthenia gravis. **Standard of care** means the treatment that the medical community thinks is appropriate and widely accepted for a condition.

Doses of rozanolixizumab were measured in milligrams per kilogram of body weight (mg/kg). This is a way of giving a dose of a drug based on a person's body weight. Each participant's dose contained a certain number of milligrams (mg) for each kilogram (kg) of their body weight.

There were 2 groups of participants who received rozanolixizumab. **Group 1** received about **7 mg/kg** of rozanolixizumab. **Group 2** received about **10 mg/kg** of rozanolixizumab.

Clinical Study Results

The doses of rozanolixizumab in milligrams that the participants in **Group 1** received could have been:

- **280 mg** if the participant weighed less than 50 kg
- **420 mg** if the participant weighed between 50 kg and 70 kg
- **560 mg** if the participant weighed between 70 kg and 100 kg
- **840 mg** if the participant weighed at least 100 kg

The doses of rozanolixizumab in milligrams that the participants in **Group 2** received could have been:

- **420 mg** if the participant weighed less than 50 kg
- **560 mg** if the participant weighed between 50 kg and 70 kg
- **840 mg** if the participant weighed between 70 kg and 100 kg
- **1,120 mg** if the participant weighed at least 100 kg

Group 3 received the placebo.

None of the participants, study doctors, or study staff knew what treatment each participant was receiving. UCB staff also did not know. The only people who knew what treatment each participant was receiving were the people who prepared the treatments. Some studies are done this way because knowing what treatment the participants are receiving can affect the results of the study. After the study was completed, UCB learned what treatment each participant received so they could create a report of the results.

The researchers used a computer program to randomly choose if the participants received rozanolixizumab or the placebo. This helped make sure the treatments were chosen fairly and comparing the results for the treatments was as accurate as possible.

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

	Group 1	Group 2	Group 3
	66 participants	67 participants	67 participants
	About **7 mg/kg** of rozanolixizumab	About **10 mg/kg** of rozanolixizumab	Placebo
	As a subcutaneous infusion		
	1 infusion on Day 1, then 1 infusion every week for 5 weeks until there was a total of 6 infusions		

What happened during this study?

This section shows how the study was planned to be done.

Before receiving study treatment, the participants visited their clinic once. All the participants first learned about the study and then decided to join. This is called **informed consent**. Then, the study doctors and study staff asked the participants about their medical history and checked their health to make sure they could join the study. This part lasted up to 4 weeks.

At this part of the study, the study doctors also:

- Did physical exams, checked the participants' vital signs, and asked about the participants' medications and any medical problems.
- Checked the participants' heart health using an electrocardiogram (ECG).
- Took blood and urine samples.
- Took pictures of the participants' chests using X-rays.
- Assessed the severity of the participants' generalized myasthenia gravis.

At this visit, the participants:

- Answered questions about their generalized myasthenia gravis, their symptoms, and their general physical and mental health.

While receiving study treatment, the participants visited the clinic between 7 and 9 times, with 1 of these visits taking place a week after they received their last dose of study treatment. This part of the study was called the **treatment period**. At most of these visits, the study doctors or staff gave the participants their study treatment. The study doctors kept track of any medical problems reported by the participants or observed by the doctors or study staff. This part lasted up to about 6 weeks.

At these visits, the study doctors also:

- Checked the participants' vital signs.
- At most visits, did physical exams and asked about the participants' medications.
- At most visits, took blood and urine samples.
- At some visits, checked the participants' heart health using an ECG.
- At some visits, assessed the severity of the participants' generalized myasthenia gravis.

At most of these visits, the participants:

- Answered questions about their generalized myasthenia gravis, their symptoms, their quality of life, and their general physical and mental health.

After the treatment period, the participants visited the clinic at least 2 times. There were also 2 other visits that could have been done online or in person. This part of the study was called the **observation period**. At these visits, the study doctors checked the participants' health and asked about any medical problems. This part lasted up to 8 weeks.

At the in-person visits, the study doctors also:

- Did physical exams, checked the participants' vital signs, and asked about the participants' medications.
- Took blood and urine samples.
- At the last visit, checked the participants' heart health using an ECG.
- At the last visit, assessed the severity of the participants' generalized myasthenia gravis.

At these visits, the participants:

- Answered questions about their generalized myasthenia gravis, their symptoms, and their general physical and mental health.

After completing the observation period, some of the participants could choose to continue receiving rozanolixizumab in another study.

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.

Did rozanolixizumab improve the participants' generalized myasthenia gravis symptoms?

Yes. The researchers found that the participants who received rozanolixizumab had improvements in their generalized myasthenia gravis symptoms compared to the participants who received the placebo.

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 completed the MG-ADL before the study and at certain points during the study. The answers the participants gave were given a score. A higher score means that a person's generalized myasthenia gravis is more severe.

The researchers compared each participant's score from before they received study treatment with their score after they had received all of their doses of study treatment. This allowed the researchers to measure a change in the participant's MG-ADL after 6 weeks of treatment.

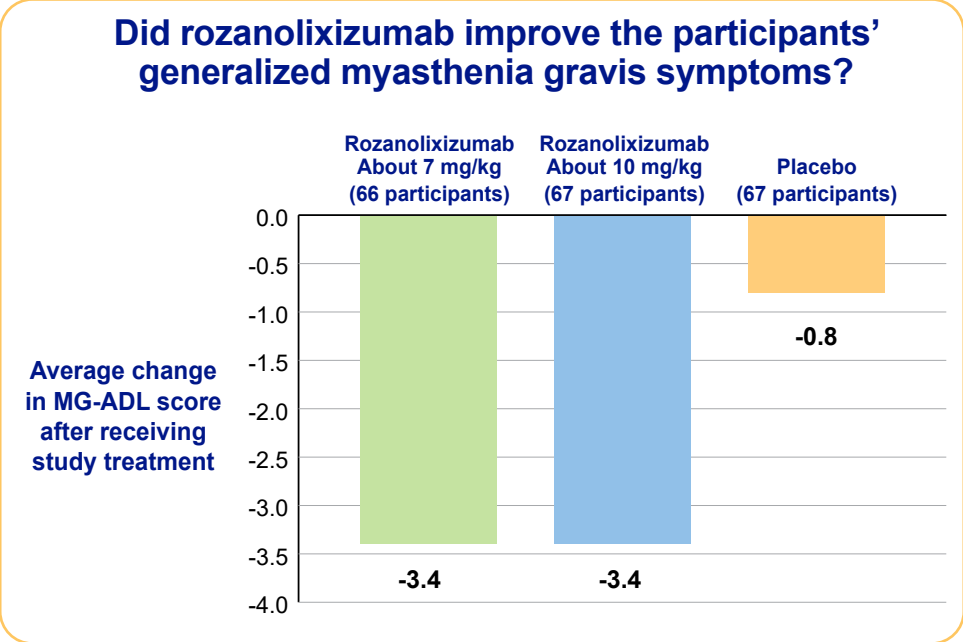
Then, the researchers calculated the average change in the MG-ADL scores for the participants in the rozanolixizumab groups and the placebo group. The researchers compared the average changes from the rozanolixizumab groups with the average change from the placebo group.

If a participant's scores had **decreased** over time, this meant that their generalized myasthenia gravis symptoms had improved.

The results from this study were:

- For the participants who received **about 7 mg/kg** of rozanolixizumab, the average change in MG-ADL score after 6 weeks of treatment was **−3.4**.
- For the participants who received **about 10 mg/kg** of rozanolixizumab, the average change in MG-ADL score after 6 weeks of treatment was **−3.4**.
- For the participants who received the **placebo**, the average change in MG-ADL score after 6 weeks of treatment was **−0.8**.

The graph below shows the results from this study.



Overall, the researchers found that the decrease in the participants' MG-ADL scores was greater in the rozanolixizumab groups compared to the placebo group. This means that the participants who received rozanolixizumab had a meaningful improvement in their generalized myasthenia gravis symptoms.

What medical problems did the study doctors report as possibly related to the 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 the study treatment. These medical problems are called **adverse reactions**.

In this study, the doctors did not know whether the participants were receiving rozanolixizumab or the placebo when the medical problems happened.

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 related to the 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.

The results below include all 200 participants who received at least 1 dose of study treatment. There were 2 participants who were meant to receive 7 mg/kg of rozanolixizumab, but instead received 10 mg/kg of rozanolixizumab. This meant Group 1 had 64 participants and Group 2 had 69 participants.

Did any adverse reactions happen during this study?

Adverse reactions in this study

	Group 1 **About 7 mg/kg of rozanolixizumab** (out of 64 participants)	**Group 2** **About 10 mg/kg of rozanolixizumab** (out of 69 participants)	**Group 3** **Placebo** (out of 67 participants)
How many participants had serious adverse reactions?	4.7% (3 participants)	2.9% (2 participants)	1.5% (1 participant)
How many participants had adverse reactions?	50.0% (32 participants)	56.5% (39 participants)	32.8% (22 participants)
How many participants left the study due to adverse reactions?	3.1% (2 participants)	4.3% (3 participants)	none

What serious adverse reactions did the participants have?

The table below shows the serious adverse reactions that happened during the study.

Serious adverse reactions during the study

Serious adverse reaction	Group 1 About 7 mg/kg of rozanolixizumab (out of 64 participants)	Group 2 About 10 mg/kg of rozanolixizumab (out of 69 participants)	Group 3 Placebo (out of 67 participants)
Chest pain	none	1.4% (1 participant)	none
Headache	none	1.4% (1 participant)	none
Inflammation of the stomach lining (Gastritis)	1.6% (1 participant)	none	none
Joint pain (Arthralgia)	1.6% (1 participant)	none	none
Muscle weakness	none	none	1.5% (1 participant)
Vomiting	1.6% (1 participant)	none	none

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 the participants in the entire study. There were other adverse reactions, but these happened in fewer participants.

Adverse reactions in 5.0% or more of the participants in the entire study

Adverse reaction	Group 1 About 7 mg/kg of rozanolixizumab (out of 64 participants)	Group 2 About 10 mg/kg of rozanolixizumab (out of 69 participants)	Group 3 Placebo (out of 67 participants)
Headache	32.8% (21 participants)	31.9% (22 participants)	17.9% (12 participants)
Diarrhea	18.8% (12 participants)	10.1% (7 participants)	9.0% (6 participants)
Fever	7.8% (5 participants)	13.0% (9 participants)	none
Nausea	4.7% (3 participants)	10.1% (7 participants)	6.0% (4 participants)

What did the researchers learn from this study?

The results of this study have helped researchers learn more about using rozanolixizumab in adults living with generalized myasthenia gravis.

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 website listed below:

- https://clinicaltrials.gov/ct2/show/study/NCT03971422
- https://www.clinicaltrialsregister.eu/ctr-search/search?query=2019-000968-18

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

Study Information

Protocol Number: MG0003

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

Full Study Title: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study Evaluating Efficacy and Safety of Rozanolixizumab in Adult Patients With Generalized Myasthenia Gravis

National Clinical Trial Number: NCT03971422

EudraCT Number: 2019-000968-18

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 26 July 2024.
The final clinical study report is dated 19 April 2022.