Clinical Study Results

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Study Sponsor:	UCB Biopharma SRL
Treatment Studied:	Rozanolixizumab
Protocol Number:	UP0106
Short Study Title:	A study to learn about the safety of rozanolixizumab in healthy people when given with a regular syringe compared with a syringe pump

Thank you

UCB thanks all the participants of this study. All the participants helped the researchers learn more about the safety of rozanolixizumab. Rozanolixizumab is also called UCB7665.

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?

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

What treatments did the participants get?

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The participants in this study got rozanolixizumab or a placebo.

The placebo was given in the same way as rozanolixizumab, but did not have any rozanolixizumab in it. The study participants, study doctors, and most of the study staff did not know whether each participant got rozanolixizumab or a placebo.

What were the results of the study?

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

- **What medical problems did the participants have during this study?**
  There were **78.1%** of participants who had medical problems during this study. This was **25 out of 32** participants.

- **What medical problems did the study doctors report as possibly related to the study treatment?**
  There were **50.0%** of participants who had medical problems that the study doctors reported as possibly being related to the study treatment. This was **16 out of 32** participants.

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

Where can I learn more about this study?

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You can find more information about this study on the websites listed on the last page. When 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 all patients, researchers do clinical studies to get information about how well the treatment works and about how safe it is.

The researchers in this study wanted to learn if healthy participants had any medical problems while getting rozanolixizumab or a placebo in 2 different ways. A **healthy participant** is someone who does not have the condition the treatment is trying to treat or any other serious health conditions. This information is important to find out if rozanolixizumab can improve the health of people living with **autoimmune diseases**, such as myasthenia gravis (MG).

In people living with autoimmune diseases, the person's immune system mistakenly attacks their own body. This causes some of the most common symptoms of autoimmune diseases such as tiredness, weakness, and inflammation. It is often not known why the immune system does this. Some autoimmune diseases, such as MG, are caused by a part of the immune system called **IgG antibodies** attacking the body's own cells and tissues.

There are treatments available for people with autoimmune diseases that are caused by IgG antibodies. But, these treatments do not work for all people living with these autoimmune diseases, and some of the treatments may also cause long-term medical problems.

Rozanolixizumab was designed to lower the levels of IgG antibodies in the blood. This means there are fewer IgG antibodies that are able to attack healthy cells and tissues in the body. Researchers hope that this can help reduce the symptoms of autoimmune disease.

Rozanolixizumab is usually given through a needle just under the skin over a short period of time, also called a **subcutaneous** (**SC**) **infusion**. This is done with a machine called an automatic **syringe pump**, also known as a syringe driver.

Researchers are interested in finding out if rozanolixizumab could also be given as an injection by hand just under the skin, also called an **SC injection**. This would allow people with autoimmune diseases to give themselves an injection with rozanolixizumab without needing a syringe pump, which might be easier and faster for them than an infusion.

In this study, the researchers wanted to learn about the safety of rozanolixizumab in healthy participants when it was given as an SC injection by hand, compared with when it was given as an SC infusion with a syringe pump.

What were the main questions studied?

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

- What medical problems did the participants have during this study?
- What medical problems did the study doctors report as possibly related to the study treatment?

Who participated in the study?

In this study, the researchers planned to include healthy participants who were 18 to 65 years old.

The study included 32 participants in the United Kingdom.

There were 11 healthy males and 21 healthy females who participated in this study. They were 20 to 62 years old when they joined.

Each participant was in the study for up to 12 weeks, but the whole study lasted for about 12 months.

The study started in April 2021 and ended in April 2022.

What treatments did the participants get?

The participants in this study got 1 dose of either rozanolixizumab or a placebo. The placebo was given in the same way as rozanolixizumab but did not have any rozanolixizumab in it. The researchers used the placebo to help make sure the effects they found in the study were actually caused by rozanolixizumab.

The study participants, study doctors, and most of the study staff did not know which treatment each participant was getting. UCB staff also did not know. The only people who knew which treatment each participant was getting were the people who prepared the treatments. Some studies are done this way because knowing which treatment the participants are getting can affect the results of the study. After the study was completed, UCB learned which treatment each participant got so they could create a report of the results.

This study had 4 groups. First, the participants were split into 2 groups by body weight. The first group included participants who weighed 35 to 49 kilograms (kg) (**lower** weight group). The second group included participants who weighed 50 kg or above (**higher** weight group).

Within each of these weight groups, the participants were also split into 2 groups to get the study treatment as either an SC infusion or an SC injection. Within each of these infusion and injection groups, the participants were then randomly split to get either rozanolixizumab or the placebo.

For the **lower** weight group, the participants were given the infusion or the injection based on the order in which they joined the study. Then, the researchers used a computer program to randomly choose if the participants got rozanolixizumab or the placebo.

For the **higher** weight group, the researchers used a computer program to randomly choose if the participants got the infusion or the injection, and if the participants got rozanolixizumab or the placebo.

The computer program helped make sure the treatments were chosen fairly and comparing the results for the treatments was as accurate as possible.

The doses of rozanolixizumab were measured in milligrams (mg).

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

	Group 1 (lower weight group)	**Group 2** (lower weight group)	**Group 3** (higher weight group)	**Group 4** (higher weight group)
	8 participants	8 participants	8 participants	8 participants
	560 mg of rozanolixizumab **or** the placebo	280 mg of rozanolixizumab **or** the placebo	560 mg of rozanolixizumab **or** the placebo	560 mg of rozanolixizumab **or** the placebo
	SC **infusion** with a syringe **pump**	SC **injection** with a syringe **by hand**	SC **infusion** with a syringe **pump**	SC **injection** with a syringe **by hand**
	1 dose	1 dose	1 dose	1 dose

What happened during this study?

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

Before joining the study, the participants visited the study centre 1 time. 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 visit, the study doctors also:


Did physical exams and asked about the participants' medications and any medical problems they were having.


Took blood and urine samples.


Checked the participants' heart health using an electrocardiogram (ECG).


Asked the participants to answer questionnaires about any signs and symptoms of a bacterial infection called tuberculosis.


Did a COVID-19 test.

After this first visit, the participants checked into the study centre for a 6-day overnight stay. This part of the study was the **treatment period**. They got their dose of the study treatment on the second day of their stay. The study doctors kept track of any medical problems reported by the participants or observed by the doctors or study staff.

During the participants' stay at the study centre, the study doctors also:

- Did physical exams and asked about the participants' medications and any medical problems they were having.
- Examined how well the participants' nervous systems were working.
- Took blood and urine samples.
- Checked the participants' heart health using an ECG.
- Depending on which treatment group the participants were in, asked the participants to answer questionnaires about the injection or infusion.
- For the participants who got the infusion, took a photo of the place on the participant's bodies where the infusion was given.
- For participants who had severe headaches, asked the participants to answer questionnaires about their headaches.
- Did COVID-19 tests.

After the treatment period, the participants visited the study centre 9 more times. The study doctors continued to keep track of any medical problems reported by the participants or observed by the doctors or study staff. This part of the study lasted just over 7 weeks.

At these visits, the study doctors also:

- Did physical exams at 1 visit, and asked about the participants' medications and any medical problems they were having.
- Took blood and urine samples.
- At some visits, checked the participants' heart health using an ECG.
- At 1 visit, did a COVID-19 test.

What were the results of the study?

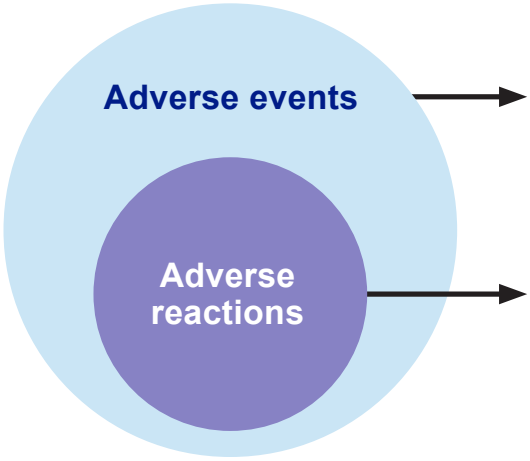
This is a summary of the main results from this study. These are the results from all the participants combined. The individual results of each participant might be different and are not in this summary.

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 related to the 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 thought may be related to the study treatment.

The information below is a summary of the **adverse events** that happened in this study.

The adverse events in the table below are shown for the participants who got the study treatment by SC infusion and for those who got it by SC injection.

	SC infusion with a syringe pump		**SC injection** with a syringe by hand	
	Rozanolixizumab (out of 12 participants)	**Placebo** (out of 4 participants)	**Rozanolixizumab** (out of 12 participants)	**Placebo** (out of 4 participants)
How many participants had **serious** adverse events?	none	none	none	none
How many participants had adverse events?	83.3% (10 participants)	25.0% (1 participant)	91.7% (11 participants)	75.0% (3 participants)
How many participants left the study due to adverse events?	none	none	none	none

None of the participants had **serious** adverse events in this study.

The most common adverse events that happened in more than 1 participant in this study were:

- Headache
- Skin redness at the place where the study treatment was given
- Fever
- Swelling at the place where the study treatment was given
- Pain at the place where the study treatment was given
- Bleeding in the vagina
- Bruising at the place where the study treatment was given
- Diarrhoea
- Itchy skin at the place where the study treatment was given
- Nausea
- Upper stomach pain
- Vomiting

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 getting rozanolixizumab or the placebo when 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.

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?

	SC infusion with a syringe pump		**SC injection** with a syringe by hand	
	Rozanolixizumab (out of 12 participants)	**Placebo** (out of 4 participants)	**Rozanolixizumab** (out of 12 participants)	**Placebo** (out of 4 participants)
How many participants had **serious** adverse reactions?	none	none	none	none
How many participants had adverse reactions?	83.3% (10 participants)	none	41.7% (5 participants)	25.0% (1 participant)
How many participants left the study due to adverse reactions?	none	none	none	none

What serious adverse reactions did the participants have?

None of the participants had **serious** adverse reactions during this study.

What adverse reactions did the participants have?

The most common adverse reactions were **headache** and **skin redness at the place where the study treatment was given**.

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

Adverse reactions in 2 or more participants in this study

	SC infusion with an automatic syringe pump		**SC injection** with a syringe by hand	
	Rozanolixizumab (out of 12 participants)	**Placebo** (out of 4 participants)	**Rozanolixizumab** (out of 12 participants)	**Placebo** (out of 4 participants)
Headache	50.0% (6 participants)	none	8.3% (1 participant)	none
Skin redness at the place where the study treatment was given	33.3% (4 participants)	none	25.0% (3 participants)	none
Fever	41.7% (5 participants)	none	none	25.0% (1 participant)
Swelling at the place where the study treatment was given	8.3% (1 participant)	none	25.0% (3 participants)	none
Pain at the place where the study treatment was given	16.7% (2 participants)	none	8.3% (1 participant)	none
Bruising at the place where the study treatment was given	16.7% (2 participants)	none	none	none
Diarrhoea	16.7% (2 participants)	none	none	none
Itchy skin at the place where the study treatment was given	none	none	16.7% (2 participants)	none
Nausea	8.3% (1 participant)	none	none	25.0% (1 participant)
Upper stomach pain	8.3% (1 participant)	none	none	25.0% (1 participant)
Vomiting	16.7% (2 participants)	none	none	none

What did the researchers learn from this study?

The results of this study have helped researchers learn more about the safety of rozanolixizumab in healthy participants.

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 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://www.clinicaltrials.gov/study/NCT04828343

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

Study Information

Protocol Number: UP0106

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

Full Study Title: A Randomized, Participant-Blind, Investigator-Blind, Placebo-Controlled Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Rozanolixizumab Administered Subcutaneously Via Manual Push Versus Syringe Driver to Healthy Participants

National Clinical Trial Number: NCT04828343

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 21 December 2023.
The final clinical study report is dated 12 December 2022.