Clinical Study Results

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Study Sponsor:	UCB Biopharma SRL
Treatment Studied:	Rozanolixizumab
Protocol Number:	TP0003
Short Study Title:	A study to learn how rozanolixizumab works in people with primary immune thrombocytopenia (ITP)

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 primary immune thrombocytopenia (**ITP**). Rozanolixizumab is also called UCB7665.

This is a summary of the main results of this study. This study is sometimes called the **myOpportunITy1** study. An independent, non-profit organization helped prepare this summary of the study results.

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 primary immune thrombocytopenia (**ITP**). 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 receive?

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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. The study participants, study doctors, and most of the study staff did not know whether a participant received 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:

- **Did rozanolixizumab increase and maintain the amount of platelets in the participants' blood?**

  Because the study was stopped early, there were too few participants and not enough information collected for researchers to confidently conclude whether or not rozanolixizumab increased and maintained the participants' platelet levels compared with the placebo.

  There were **19.0%** of participants who received rozanolixizumab who had a maintained increase in their platelet levels. This was **4 out of 21** participants. There were **no** participants who received the placebo who had a maintained increase in their platelet levels. This was **0 out of 12** participants.

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

  71.4% of participants who received **rozanolixizumab** had medical problems that the study doctors reported as possibly being related to the study treatment. This was **15 out of 21** participants.

  25.0% of participants who received the **placebo** had medical problems that the study doctors reported as possibly being related to the study treatment. This was **3 out of 12** 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. If a full report of the study results is available, it also can be found on these 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 rozanolixizumab worked in participants living with ITP. They also wanted to learn if the participants had any medical problems during the study.

Primary immune thrombocytopenia (**ITP**) is a rare condition of the blood that affects a part of the blood called **platelets**. Platelets help with healing wounds. Blood travels around the body through **blood vessels** such as veins, arteries, and capillaries. When a blood vessel is damaged, blood leaks from the wound, which is called **bleeding**. Platelets help stop bleeding by forming a clot where the blood vessel is damaged. This helps the blood vessel to heal.

In people with ITP, the body's own immune system mistakenly attacks platelets and destroys them. Because of this, people with ITP usually have very low levels of platelets in their blood. This is known as **thrombocytopenia**.

Because of their very low platelet levels, people with ITP often bleed or bruise more easily and take longer to stop bleeding than people with healthy platelet levels. Increased bleeding also leads to small spots of bleeding under the skin that often look like small bruises. These are called **petechiae** if the blood spot is small, or **purpura** if the blood spot is large. In serious cases, people with ITP can experience a lot of blood loss.

There are treatments available for people with ITP. But these treatments do not work for all people with ITP, and some people may not be able to receive them at all.

In people with ITP, a part of the immune system called **IgG antibodies** attach to platelets and cause them to be destroyed. The study treatment, **rozanolixizumab**, was designed to lower the amount of IgG antibodies in the blood. This means there are fewer IgG antibodies that can attach to platelets in people with ITP. This may help to stop the platelets from being attacked and destroyed by the immune system. Researchers hope that rozanolixizumab can help increase and maintain the platelet levels in people with ITP.

In this study, the researchers wanted to learn if rozanolixizumab increased and maintained the platelet levels of people with ITP compared with a placebo. 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 increase and maintain the amount of platelets in the participants' blood?
- What medical problems did the study doctors report as possibly related to the study treatments?

Who participated in the study?

There were 10 males and 23 females with ITP who participated in this study. They were 18 to 78 years old when they joined.

The study included participants in 12 countries:

Country	Participants	Country	Participants
France	1	Poland	10
Georgia	4	Russia	1
Hungary	3	South Korea	1
Italy	1	Taiwan	1
Japan	3	Ukraine	5
Moldova	2	The United Kingdom	1

In this study, the researchers planned to include participants living with ITP who:

- Had been diagnosed with ITP for at least 3 months before the start of the study
- Had very low levels of platelets when they joined the study
- Had previously responded to a standard treatment for ITP
- Had previously been treated with at least 2 other standard treatments for ITP, but the treatments had stopped working, didn't work at all, or the participant had too many side effects

Most of the participants could be in the study for up to 34 weeks. If a participant had their spleen removed before joining this study, then they could be in the study for up to 48 weeks.

Some people with ITP have their spleens removed to help treat ITP. This is because the spleen is where most of the platelet destruction happens and is often where IgG antibodies are made. But the spleen plays an important role in the immune system, so people who have had their spleen removed need to have certain vaccinations to help protect them from infections. The study doctors checked whether these participants had received all of the required vaccinations and gave them the vaccinations if they had not. This was done before the participants started receiving their study treatments, and is why these participants were in the study for a longer time.

The whole study lasted for just over 2 years.

The study started in January 2020 and ended in April 2022.

The sponsor stopped the study early because other treatment options were made available to treat people with ITP while this study was happening. So, the sponsor decided not to continue researching rozanolixizumab in people with ITP. But all of the participants who had already joined this study were allowed to finish the study as planned.

What treatments did the participants receive?

The participants in this study received rozanolixizumab or a placebo over a period of time through a needle just under their skin, also called a **subcutaneous infusion.**

This study had 2 groups. The participants in **Group 1** received rozanolixizumab. The participants in **Group 2** received 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 infusions to help make sure the effects they found in the study were actually caused by rozanolixizumab.

This study was a **double-blind** study. This means that the study participants, study doctors, and most of the study staff did not know which treatment each participant was receiving. UCB staff also did not know. The only people who knew which treatment each participant was receiving were the people who prepared the treatments. Some studies are done this way because knowing which treatment the participants are receiving can affect the results of the study. After the study was completed, UCB learned which 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 is called randomization. The researchers also measured the participants' platelet levels at the beginning of the study and checked whether their spleen had been removed. These factors were included in the computer program that randomized the participants. Randomization 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. The doses of rozanolixizumab that a participant received depended on their body weight. The doses of rozanolixizumab could have been reduced if the study doctors thought it was necessary.

	Group 1 Rozanolixizumab	Group 2 Placebo
	21 participants	12 participants
	One higher dose followed by lower doses of rozanolixizumab	The placebo
	As infusions under the skin	
	Once every 2 weeks for 24 weeks	

What happened during this study?

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

Before joining the study, most of the participants visited their clinic 1 time. If a participant had their spleen removed before the study, they visited their clinic up to 7 times before joining the study. 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. For most of the participants, this part lasted up to 4 weeks. For the participants whose spleen had been removed, this part lasted up to 18 weeks. During this part of the study, the doctors also:

Did physical exams and asked about the participants' medications

Took blood and urine samples

Checked some of the participants' chests with an X-ray for signs of a bacterial infection called tuberculosis

Asked the participants to answer questionnaires about any signs and symptoms of tuberculosis

Checked how well the participants were able to do their usual daily activities

Assessed the severity of the participants' bleeding

For the participants whose spleen had been removed, checked whether they had all the required vaccinations and gave them the vaccinations if they did not, which is why this part of the study took 18 weeks for those participants

During the study, the participants visited the clinic 26 times. The study doctors kept track of any medical problems reported by the participants or observed by the doctors or study staff. This part of the study lasted up to 24 weeks. At these visits, the study doctors also:

- Did physical exams at most visits, and asked about the participants' medications
- Took blood and urine samples
- At most visits, assessed the severity of the participants' bleeding
- At some visits, checked the participants' heart health using an electrocardiogram (ECG)
- At some visits, asked the participants to answer questionnaires about their overall health and any signs and symptoms they may be experiencing
- At some visits, gave the participants their study treatment

After the 24-week treatment period, some participants joined another study. The participants who did not join the other study visited their study site 3 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 up to 6 weeks. At these visits, the study doctors also:

- Did physical exams at most visits, and asked about the participants' medications
- Took blood and urine samples
- Assessed the severity of the participants' bleeding
- At most visits, checked some of the participants' chests with an X-ray for signs of tuberculosis
- At most visits, asked the participants to answer questionnaires about their overall health and any signs and symptoms they may be experiencing
- At some visits, checked the participants' heart health using an ECG

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 increase and maintain the amount of platelets in the participants' blood?

To try to answer this question, the researchers measured the amount of platelets in the participants' blood:

- At the beginning of the study
- While the participants received the study treatments
- After the participants received the study treatments

Using these measurements, the researchers then calculated the number of participants who had a **clinically meaningful increase** in their platelet levels. In this study, a clinically meaningful increase meant that the participants' platelet levels had risen above a certain amount in the blood for a certain period of time while receiving the study treatments.

For it to be considered a clinically meaningful increase, a participant had to have high enough platelet levels for at least 8 out of the last 12 weeks of receiving their study treatment.

During the last 12 weeks of treatment:

- **19.0%** of the participants who received **rozanolixizumab** had a clinically meaningful increase in their platelet levels. This was **4 out of 21** participants.
- **None** of the participants who received **the placebo** had a clinically meaningful increase in their platelet levels. This was **0 out of 12** participants.

Because the study was stopped early, there were too few participants and not enough information collected for researchers to confidently conclude whether or not rozanolixizumab increased and maintained the participants' platelet levels compared with the placebo.

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

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 treatments. 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 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 treatments. 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?

Adverse reactions in this study

Adverse reaction	Group 1 Rozanolixizumab (out of 21 participants)	Group 2 Placebo (out of 12 participants)
How many participants had serious adverse reactions?	none	none
How many participants had adverse reactions?	71.4% (15 participants)	25.0% (3 participants)
How many participants left the study due to adverse reactions?	4.8% (1 participant)	none

What serious adverse reactions did the participants have?

There were no serious adverse reactions in this study. 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 2 or more participants in the entire study. There were other adverse reactions, but those happened in only 1 participant each.

Adverse reactions in 2 or more participants in the entire study

Adverse reaction	Group 1 Rozanolixizumab (out of 21 participants)	Group 2 Placebo (out of 12 participants)
Headache	61.9% (13 participants)	25.0% (3 participants)
Fever	28.6% (6 participants)	none
Vomiting	19.0% (4 participants)	none
Nausea	14.3% (3 participants)	none
An increase in body temperature	9.5% (2 participants)	none
Diarrhea	4.8% (1 participant)	8.3% (1 participant)

What did the researchers learn from this study?

The results of this study have helped researchers learn more about using rozanolixizumab in people living with ITP.

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 ITP with rozanolixizumab were not 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/NCT04200456
- www.clinicaltrialsregister.eu/ctr-search/search?query=2019-000884-26

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

Study Information

Protocol Number: TP0003

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

Full Study Title: A Phase 3 Multicenter, Double-Blind, Randomized, Placebo-Controlled Study to Evaluate the Efficacy, Safety, and Tolerability of Rozanolixizumab in Adult Study Participants With Persistent or Chronic Primary Immune Thrombocytopenia (ITP)

National Clinical Trial Number: NCT04200456

EudraCT Number: 2019-000884-26

IND Number: 143499

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 13 December 2023.
The final clinical study report is dated 21 March 2023.