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Study Sponsor:	UCB Biopharma SRL
Treatment Studied:	Rozanolixizumab
Protocol Number:	TP0004
Short Study Title:	A study to learn about the safety of rozanolixizumab 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 the safety of 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 **myOpportunITy3** 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.

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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 treatment did the participants receive?

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The participants in this study received rozanolixizumab.

What were the results of the study?

Page 9

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

- **What medical problems did the participants have during this study?**
  There were **90.7%** of participants who had medical problems during this study. This was **39 out of 43** participants. The most common medical problem was **headache**. There were **41.9%** of participants who had headache. This was **18 out of 43** participants.

- **What medical problems did the study doctors report as possibly related to the study treatment?**
  There were **58.1%** of participants who had medical problems that the study doctors reported as possibly being related to the study treatment. This was **25 out of 43** participants. The most common medical problem that the study doctors reported as possibly being related to the study treatment was **headache**. There were **34.9%** of participants who had headache that doctors reported as possibly related to the study treatment. This was **15 out of 43** 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 can also be found on these websites.

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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 the participants living with ITP had any medical problems while receiving rozanolixizumab.

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 about the safety of rozanolixizumab in people with ITP.

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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?

There were 15 males and 28 females with ITP who participated in this study. They were 18 to 78 years old when they joined.

The study included participants in 14 countries:

Country	Participants	Country	Participants
China	5	Russia	2
Georgia	2	Spain	3
Germany	1	Taiwan	2
Hungary	2	Ukraine	10
Italy	1	The United Kingdom	1
Japan	1	The United States of America	1
Moldova	1		
Poland	11		

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

- had taken part in and completed 1 of 2 other studies of rozanolixizumab for their ITP before starting this study

Each participant could be in the study for up to just over 1 year, but the whole study lasted for about 2 years.

The study started in January 2021 and ended in December 2022.

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The sponsor stopped the study early because other treatment options were made available to treat people with ITP while this study was happening. As a result, the sponsor decided not to continue researching rozanolixizumab in people with ITP. Participants who had already joined this study were allowed to finish the study as planned if they joined before December 2021. The participants who joined later were allowed to continue in the study until December 2022.

What treatment did the participants receive?

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

All of the participants in this study had completed 1 of 2 other studies of rozanolixizumab for their ITP before starting this study. These 2 studies were **TP0003** and **TP0006** and are known as **feeder studies**. As soon as the participants completed these feeder studies, they started receiving rozanolixizumab as part of this study.

Participants who received rozanolixizumab during the feeder studies continued to receive rozanolixizumab at the same dose for this study. The study doctors could choose to increase or decrease this dose depending on the participants' platelet levels at the start of this study.

Participants who received the placebo during the feeder studies started receiving rozanolixizumab as part of this study.

During this study, the study researchers changed how often some of the participants received a dose of rozanolixizumab. If the study doctors and the participants agreed, some participants changed from a dose of rozanolixizumab every 2 weeks to a dose of rozanolixizumab every week. If not, the participants left the study.

This means that the participants received either:

- A dose of rozanolixizumab every 2 weeks for the entire time they were in the study
- A dose of rozanolixizumab every week for the entire time they were in the study
- A dose of rozanolixizumab every 2 weeks and then changed during the study to receive a dose every week instead

This study was an **open-label** study. This means that the participants, study doctors, study staff, and UCB staff knew that all the participants in this study were receiving rozanolixizumab.

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The chart below shows the treatments the researchers planned to study. The participants could receive either high, medium, or low doses of rozanolixizumab. The doses of rozanolixizumab that a participant received during this study could have been increased or decreased between these 3 doses depending on the participant's platelet levels.

	Rozanolixizumab
	43 participants
	Either high, medium, or low doses of rozanolixizumab
	As infusions just under the skin
	For 20 participants: Once every 2 weeks For 1 participant: Once every week For 22 participants: Once every 2 weeks and then once every week

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What happened during this study?

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

Before joining this study, the participants took part in and completed 1 of the 2 feeder studies of rozanolixizumab for their ITP. At the last visit of the feeder studies, the participants learned about this study and then decided to join. This is called **informed consent**. Then, the study doctors and study staff checked their health to make sure they could join this study. This visit took place up to 7 days after the participants completed their feeder study. At this visit, the study doctors also:

Did physical exams and asked about the participants' medications

Took blood and urine samples

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

Asked the participants to answer questionnaires about their overall health and any signs and symptoms they may be experiencing

Assessed the severity of the participants' bleeding

Gave the participants their first dose of rozanolixizumab in this study

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After the first visit of this trial, the participants visited their clinic another 53 times. This part of the study was the **treatment period** and lasted up to 52 weeks. The study doctors kept track of any medical problems reported by the participants or observed by the doctors or study staff. At these visits, the study doctors also:


Did physical exams and asked about the participants' medications


At most visits, took blood samples


At some visits, took urine samples


At most visits, assessed the severity of the participants' bleeding


At some visits, checked the participants' heart health using an ECG


At some visits, asked the participants to answer questionnaires about their overall health and any signs and symptoms they may be experiencing


At most visits, gave the participants rozanolixizumab

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After the treatment period, the participants visited their clinic 2 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 7 weeks. At these visits, the study doctors also:


Did physical exams at the final visit, and asked about the participants' medications


Took blood samples


At the final visit, took urine samples


Assessed the severity of the participants' bleeding


Asked the participants to answer questionnaires about their overall health and any signs and symptoms they may be experiencing


At the final visit, 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.

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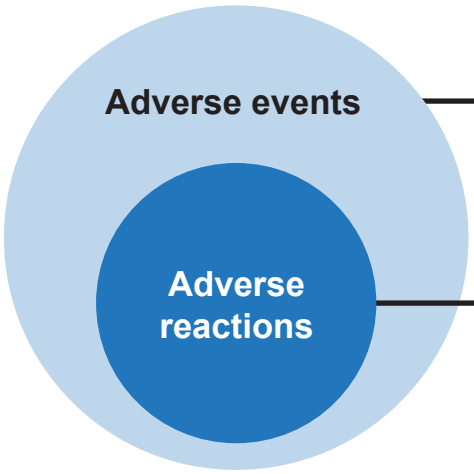
What medical problems did the participants have during this study?

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

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

	Rozanolixizumab (out of 43 participants)
How many participants had serious adverse events?	20.9% (9 participants)
How many participants had adverse events?	90.7% (39 participants)
How many participants left the study due to adverse events?	none

The serious adverse events were:

- Abnormal bleeding from the uterus
- COVID-19 infection
- A break in a bone in the forearm called the radius (Radius fracture)
- A non-cancerous tumor in a muscle (Leiomyoma)
- Bleeding
- Bleeding from the skin
- Decreased levels of platelets in the blood
- Dislocation of a joint
- Fever
- Low number of platelets in the blood due to an immune reaction (Immune thrombocytopenia)
- Small spots of bleeding under the skin (Purpura)

The most common adverse events that happened in 10% or more of participants in the entire study were:

- Headache
- Fever
- COVID-19 infection
- An increase in body temperature
- Diarrhea
- Feeling tired (Fatigue)
- Nausea

There were no adverse events that caused the participants to leave the study.

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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**.

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?

Adverse reactions in this study

	Rozanolixizumab (out of 43 participants)
How many participants had serious adverse reactions?	2.3% (1 participant)
How many participants had adverse reactions?	58.1% (25 participants)
How many participants left the study due to adverse reactions?	none

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What serious adverse reactions did the participants have?

There was 1 serious adverse reaction in this study. This serious adverse reaction was **fever** and happened in 2.3% of participants in this study. This was 1 out of 43 participants. The participant had mild fever but was admitted to the hospital because the doctors thought the participant had COVID-19. It was later confirmed that the participant did not have COVID-19.

None of the participants died due to serious adverse reactions.

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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 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

Adverse reaction	**Rozanolixizumab** (out of 43 participants)
Headache	34.9% (15 participants)
Fever	16.3% (7 participants)
Diarrhea	11.6% (5 participants)
An increase in body temperature	9.3% (4 participants)
Nausea	9.3% (4 participants)
Vomiting	7.0% (3 participants)
Feeling tired (Fatigue)	4.7% (2 participants)
Infection of the nose and throat (Upper respiratory tract infection)	4.7% (2 participants)
Muscle aches and pain (Myalgia)	4.7% (2 participants)
Rash	4.7% (2 participants)

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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.

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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/NCT04596995
- www.clinicaltrialsregister.eu/ctr-search/search?query=2019-000883-40

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

Study Information

Protocol Number: TP0004

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 Investigate the Long-Term Safety, Tolerability, and Efficacy of Rozanolixizumab in Study Participants With Persistent or Chronic Primary Immune Thrombocytopenia

National Clinical Trial Number: NCT04596995

EudraCT Number: 2019-000883-40

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.

This summary was last updated on 19 December 2023.
The final clinical study report is dated 20 November 2023.

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