Clinical Study Results

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Study Sponsor: UCB Biopharma SRL

Drug Studied: Rozanolixizumab

Protocol Number: AIE001

Short Study Title: A study to learn if rozanolixizumab works in people with LGI1 autoimmune encephalitis (AIE) and to learn about its safety

Thank you

UCB thanks all the participants and caregivers of this study. All the participants helped the researchers learn more about using rozanolixizumab in people living with **LGI1 autoimmune encephalitis**, also called LGI1 AIE.

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 09 04 2025. The information in this summary is current as of this date.

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Overview of this study

Why was the research needed?

Researchers are looking for a way to treat LGI1 AIE. 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 treatments did the participants receive?

The participants in this study received either rozanolixizumab or a placebo. A placebo looks like a drug but does not have any medicine in it.

What were the results of this study?

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

- **Did rozanolixizumab help reduce seizures caused by LGI1 AIE?**

  The number of participants in this study was too small for the researchers to know if rozanolixizumab helped reduce seizures caused by LGI1 AIE. The differences between the rozanolixizumab group and the placebo group could have happened by chance.

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

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

More information about the medical problems that happened in the study is included later in this summary.

There were 33% of participants (4 out of 12) who had medical problems that the study doctors reported as **possibly being related** to study treatment. This was:

- 17% of participants (1 out of 6) who received **rozanolixizumab** during this study.
- 50% of participants (3 out of 6) who received the **placebo** during this study.

Where can I learn more about this study?

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.

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Why was the research needed?

The researchers in this study wanted to learn if rozanolixizumab worked in a small number of participants living with LGI1 AIE. They also wanted to learn if the participants had any medical problems during the study.

Autoimmune diseases are caused by problems with the immune system. The immune system is the body's natural defense system against viruses and bacteria. In people with autoimmune diseases, the immune system attacks healthy tissues in the body by mistake.

Autoimmune encephalitis (**AIE**) is an autoimmune disease that causes inflammation in the brain. People with AIE often have symptoms such as:

- Memory loss
- Difficulty sleeping
- Reduced thinking abilities
- Seizures
- Other medical problems

There are several types of AIE. The researchers in this study focused on a type of AIE called leucine-rich glioma-inactivated 1 (**LGI1**) AIE. In people with LGI1 AIE, proteins in the immune system called **IgG autoantibodies** attack the LGI1 protein in the brain. This leads to inflammation in the brain.

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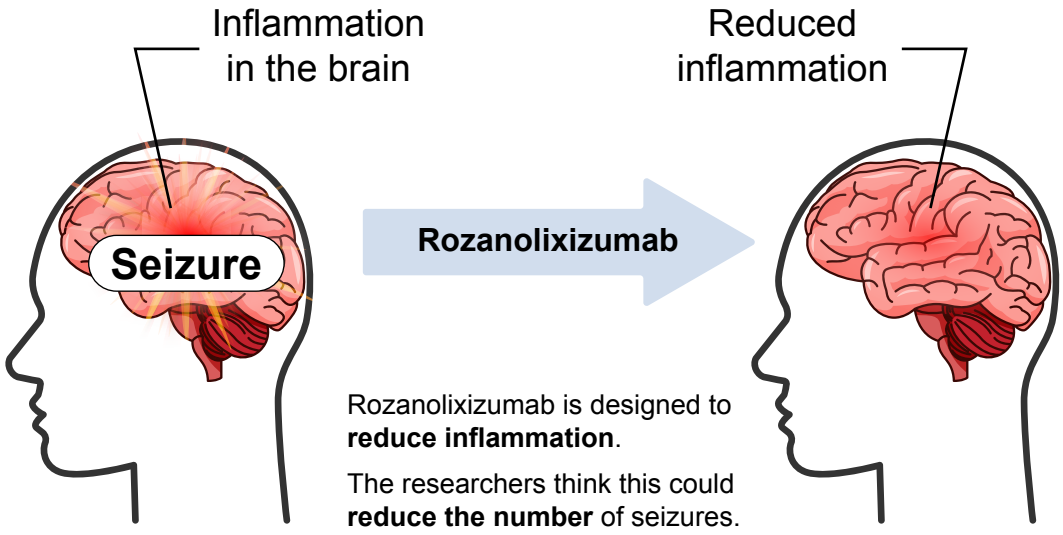
People with LGI1 AIE are very likely to have seizures caused by inflammation. **Seizures** are episodes of uncontrolled electrical activity in the brain. They can last a long time or for only a few seconds. The seizures can affect different parts of the brain. Some types of seizures happen up to 100 times in a single day. When a person with AIE has seizures, other symptoms like memory loss can become worse.

The study drug **rozanolixizumab** is designed to reduce the levels of the IgG autoantibodies that cause inflammation. Rozanolixizumab is already used to treat other autoimmune diseases. The researchers in this study wanted to know if rozanolixizumab could help reduce seizures caused by inflammation in people with LGI1 AIE.



What was the main question studied?

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

- **Did rozanolixizumab help reduce seizures caused by LGI1 AIE?**

The researchers also wanted to know about any medical problems that were possibly related to study treatment.

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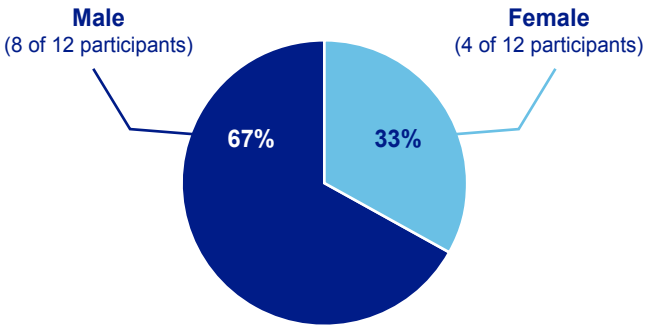
Who participated in the study?

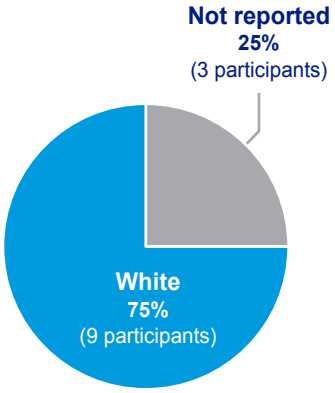
There were 12 participants with LGI1 AIE who participated in this study. They were 41 to 84 years old when they joined.

Sex of participants



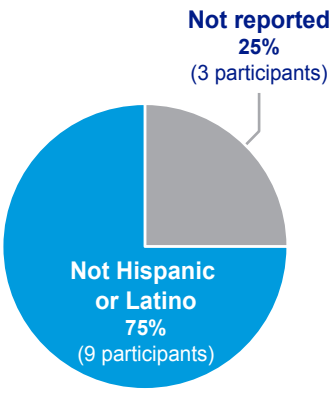
Percentage of participants (%)

Race
(out of 12 participants)



Percentage of participants (%)

Ethnicity
(out of 12 participants)



Percentage of participants (%)

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Clinical Study Results

The study included participants in 7 countries.

This study included participants living with LGI1 AIE who:

- Had AIE for less than 1 year before joining the study.
- Were recently having at least 2 seizures per week.
- Had someone who could help them during the whole study.
- Had received a high dose of corticosteroids to treat inflammation in the brain caused by LGI1 AIE within 6 weeks of joining the study. Corticosteroids are treatments that help reduce inflammation in the body. High doses of corticosteroids can be used for a short period of time to treat severe inflammation.

Each participant who completed the study was in the study for up to 10 months. The whole study lasted 2 years and 7 months. The study started in September 2021 and ended in April 2024.

The study was designed to include 68 participants. But, the sponsor ended the study early because they could not find enough participants with LGI1 AIE who could join within the study's defined timelines.

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What treatments did the participants receive?

The participants in this study received rozanolixizumab or a placebo as injections just under the skin. The placebo injections did not have any rozanolixizumab in them. The researchers used the placebo to better understand what effects may have been related to rozanolixizumab.

In this summary, “study treatment” means anything the participants received as a part of the study. This includes rozanolixizumab and the placebo. **Rozanolixizumab** is the drug that the researchers wanted to learn more about.

None of the participants, caregivers, study doctors, or study staff knew what treatment each participant was receiving. UCB staff also did not know. 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 studied:

	Rozanolixizumab	Placebo
	6 participants	6 participants
	Rozanolixizumab through a needle just under the skin over time	The placebo through a needle just under the skin over time
	Once a week for 24 weeks	

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What happened during this study?

All the participants and their caregivers 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:

Before study treatment

1 site visit

The study doctors checked the health of the participants to make sure they could join the study. To do this, they asked the participants about their symptoms and medical problems and did different tests and measurements. These tests and measurements included:

- blood draws
- an MRI scan
- for participants who volunteered, a lumbar puncture (spinal tap), which is a procedure to collect fluid around the spinal cord

The study doctors also asked the participants and their caregivers to start recording seizures in a diary and answer questionnaires about the participants’ symptoms and quality of life.

Up to 6 weeks before starting study treatment

During study treatment

Up to 9 site visits, and 18 other visits which could be done at home, by phone, or at the study site

- The study doctors checked the health of the participants with different tests and measurements, including blood draws. They also asked the participants about their symptoms and any medical problems.
- The participants received their study treatment.
- The participants and their caregivers kept track of seizures in a diary and answered questionnaires about the participants’ symptoms and quality of life.

24 weeks

After study treatment

2 site visits and 1 phone visit

The study doctors checked the health of the participants with different tests and measurements, including:

- blood draws
- for participants who volunteered, a lumbar puncture (spinal tap), which is a procedure to collect fluid around the spinal cord

The study doctors also asked the participants about their symptoms and any medical problems.

The participants and their caregivers answered questionnaires about the participants’ symptoms and quality of life.

Up to 8 weeks after finishing study treatment

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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 help reduce seizures caused by LGI1 AIE?

To answer this question, the researchers looked at the participants' seizure diaries and counted the number of participants who stayed "**seizure-free**" until the end of their treatment. This meant going 4 weeks in a row without having any seizures **and** continuing to have no seizures until the end of their treatment.

After 24 weeks of treatment, the researchers found that:

- **None** of the 6 participants who received **rozanolixizumab** during this study stayed seizure-free until the end of their treatment.
- **1 out of 6** participants who received the **placebo** during this study stayed seizure-free until the end of their treatment. This was 17% of the participants in this group.

Because there were very few participants in this study, the researchers could not determine whether or not rozanolixizumab helped reduce seizures caused by LGI1 AIE. The differences between the rozanolixizumab group and the placebo group could have happened by chance.

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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**".

In this study, the doctors did not know what the participants were receiving when the medical problems happened. The study doctors reported the medical problems they thought were caused by the study drug, even though the participants could have received the placebo. So, some adverse reactions may be reported in participants who received the placebo, even though the placebo does not directly cause medical problems.

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.

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Did any adverse reactions happen during this study?

There were 33% of participants (4 out of 12) who had an adverse reaction in this study.

Adverse reactions in this study

	Rozanolixizumab (out of 6 participants)	**Placebo** (out of 6 participants)
How many participants had adverse reactions?	17% (1 participant) 	50% (3 participants) 
How many participants had **serious** adverse reactions?	17% (1 participant) 	none 
How many participants left the study due to adverse reactions?	17% (1 participant) 	none 

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What adverse reactions did the participants have?

The table below shows all the adverse reactions that happened in this study.

Some participants had more than 1 adverse reaction:

- The adverse reactions in the rozanolixizumab group all happened in the same participant.
- The adverse reactions in the placebo group happened in 3 different participants.

Adverse reaction	**Rozanolixizumab** (out of 6 participants)	**Placebo** (out of 6 participants)
Feeling weak or lacking energy (Asthenia)	17% (1)	none
Diarrhea	17% (1)	none
Stomach pain	17% (1)	none
Falling down	17% (1)	none
Feeling tired (Fatigue)	17% (1)	none
Difficulty controlling reactions or impulses	17% (1)	none
Swelling in the hands, lower legs, or feet caused by fluid buildup	17% (1)	none
Thinking about suicide (Suicidal ideation)	17% (1)	none
Vomiting	17% (1)	none
A rash with flat areas and small bumps (Rash maculopapular)	none	17% (1)
High levels of a protein that is produced by abnormal cells in the bone marrow that have the potential to develop into cancer in the future (Paraproteinemia)	none	17% (1)
A condition that causes itchy, swollen skin (Eczema)	none	17% (1)

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What serious adverse reactions did the participants have?

The serious adverse reaction that happened during the study was thinking about suicide (Suicidal ideation). This serious adverse reaction happened in 1 participant who was receiving rozanolixizumab.

None of the participants died due to serious adverse reactions.

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 LGI1 AIE.

In this study, the researchers found that:

- The number of participants was too small to know whether or not rozanolixizumab helped reduce seizures caused by LGI1 AIE.
- None of the 6 participants who received rozanolixizumab during this study stayed seizure-free until the end of their treatment.
- 17% of participants (1 out of 6) who received **rozanolixizumab** during this study had medical problems that the study doctors reported as possibly being related to study treatment.
- 50% of participants (3 out of 6) who received the **placebo** during this study had medical problems that the study doctors reported as possibly being related to study treatment.

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 AIE 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 on the websites listed below:

- https://clinicaltrials.gov/study/NCT04875975
- https://www.clinicaltrialsregister.eu/ctr-search/search?query=2019-004778-25

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

Study Information

Protocol Number: AIE001

National Clinical Trial Number: NCT04875975

EudraCT Number: 2019-004778-25

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

Full Study Title: A Randomized, Double-Blind, Placebo-Controlled, Multicenter, Phase 2 Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Rozanolixizumab in Adult Study Participants With Leucine-Rich Glioma Inactivated 1 Autoimmune Encephalitis

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 09 April 2025.
The final clinical study report is dated 25 November 2024.

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