NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn about the effects and safety of ligelizumab (QGE031) in adolescents with chronic spontaneous urticaria (CSU)

Protocol number: CQGE031C2202

Thank You!

Novartis sponsored this trial and believes it is important to share what was learned from the results of this trial with the participants and the public. Novartis would like to thank all the adolescents and their parents/guardians for taking part in this trial for the drug ligelizumab, also known as QGE031. You helped researchers learn about the effects and safety of ligelizumab in adolescents with chronic spontaneous urticaria, also known as CSU.

This summary only shows the results of a single clinical trial. Other clinical trials may have different findings. Researchers and health authorities, such as the Food and Drug Administration (FDA) in the United States and the European Medicines Agency (EMA) in Europe, look at the results of many clinical trials to understand which drugs work and if they are safe. Websites listed at the end of the summary may have more information about this trial. If you have any questions about these trial results, please talk to the doctor or staff at your trial site.

Glossary

Adverse events

medical problems that happen during clinical trials

Chronic spontaneous urticaria

when itch and hives occur several times a week for over 6 weeks or more

Clinical trial

a research investigation in which people voluntarily participate to evaluate the effects and safety of new drugs

Double-blind trial

a type of clinical trial in which the trial participants, trial doctors, and trial staff do not know what medicine the participants are receiving

Immunoglobulin

immunoglobulins are protein molecules produced by white blood cells

Ligelizumab

a drug that is developed to prevent CSU symptoms; it is given via injection under the skin

Placebo

a dummy medicine which looks like the trial drug but does not have any medicine in it

Randomization

a process of randomly assigning participants in treatment groups

Rescue medicine

fast-acting medicine given to relieve CSU symptoms immediately

Researchers

someone who conducts experiments; in this trial, these include the hospital doctor and trial staff

Trial drug

a drug being studied or researched in a clinical trial

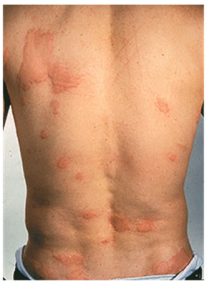
White blood cells

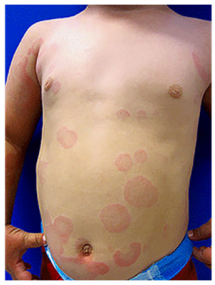
the cells of the immune system that help fight infections by attacking bacteria, viruses, and germs that attack the human body

Why was the research needed?

Itch and hives

Researchers were looking for a better way to treat itch and hives, also known as urticaria in adolescents. When it occurs several times in a week for more than 6 weeks, it is called chronic spontaneous urticaria (CSU).





Site reference: https://www.aad.org/public/diseases/a-z/hives-symptoms

In this trial, researchers wanted to learn about the effects and safety of ligelizumab in adolescents from 12 to less than 18 years of age, including those adolescents who turn 18 during the study with CSU.

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

Did the participants show improvements in the signs and symptoms of their itch and hives after 24 weeks of treatment as compared to the start of the trial?

The other questions researchers wanted to explore in this trial were:

- How many participants' itch and hives completely disappeared after treatment?
- Did participant's quality of life improve after treatment?

Trial drugs

The drugs given in this trial were:

Ligelizumab (QGE031), is the trial drug which is given as an injection and is being tested for the treatment of CSU in adolescents.

Placebo, looked like the trial drug but did not have any medicine in it. Using a placebo helps researchers better understand the effect of a trial drug by making sure that the changes were not happening by chance.

Throughout the trial, the participants continued to take their regular CSU medicine called antihistamines. Participants were also given extra doses of antihistamines to manage episodes of CSU, if needed. This medicine is known as “rescue” medicine.

Who was in this trial?

The participants could take part in this trial if they:

- were between 12 to 18 years of age
- had CSU longer than 6 months
- had CSU that could not be managed with other treatment
- did not have any other skin disease

A total of 49 participants from 20 centers across 10 countries participated in this trial.

Number of participants in each country

Average age
15 years
Range
12 to 17 years

Girls: **57%**
(28 out of 49 participants)
Boys: **43%**
(21 out of 49 participants)

Race
White: **78%** (38 out of 49)
Asian: **20%** (10 out of 49)
Black: **2%** (1 out of 49)

How was this trial done?

Before Treatment

At the start of the trial, the trial doctors checked if participants could take part in the trial.

During Treatment

None of the trial participants, trial doctors, or trial staff knew what treatment participants were receiving.

Eligible participants were randomly assigned to the following 3 groups:

Group 1:
Ligelizumab 24 mg

24 participants

Group 2:
Ligelizumab 120 mg

13 participants

Group 3:
Placebo followed by ligelizumab 120 mg

12 participants

Participants in Group 1 (Ligelizumab 24 mg) and Group 2 (Ligelizumab 120 mg) received 1 injection under the skin every 4 weeks from Day 1 to Week 20.

Participants in Group 3 received 1 injection of placebo at Day 1, Week 4 and Week 8 followed by ligelizumab 120 mg at Week 12, Week 16, and Week 20.

After Treatment

No trial drugs were given to the participants during this 16 weeks period. However, participants continued to take their regular CSU medicines. During this period, it was monitored if participants CSU came back.

Researchers closely monitored the overall health of the participants throughout the trial. Researchers completed this trial as planned.

What were the main results of this trial?

Did the participants show improvements in the signs and symptoms of their itch and hives after 24 weeks of treatment as compared to the start of the trial?

Participants

eDiary

Participants recorded their itch and hives scores twice a day on a scale of 0 (none) to 3 (severe)

Participants in all the 3 groups showed reduction in the signs and symptoms of their CSU. (**Group 1: Ligelizumab 24 mg, Group 2: Ligelizumab 120 mg, Group 3: Placebo followed by ligelizumab 120 mg**).

What were the other results of this trial?

How many participants' itch and hives completely disappeared after treatment?

Did the participants' quality of life improve after treatment?

Participants were asked to fill a questionnaire called the Children's Dermatology Life Quality Index (CDLQI) to know the impact of skin disease on quality of life (QoL) in adolescents.

Researchers noted that the quality of life improved in all 3 groups at Week 24.

What medical problems did the participants have during the trial?

Medical problems that happen in clinical trials are called "adverse events".

A lot of research is needed to know whether a drug causes an adverse event. During a trial, all adverse events are recorded, whether or not they are thought to be caused by the trial drug. When new drugs are being studied, researchers keep track of all adverse events participants have.

This section is a summary of the adverse events that happened during this trial. The websites listed at

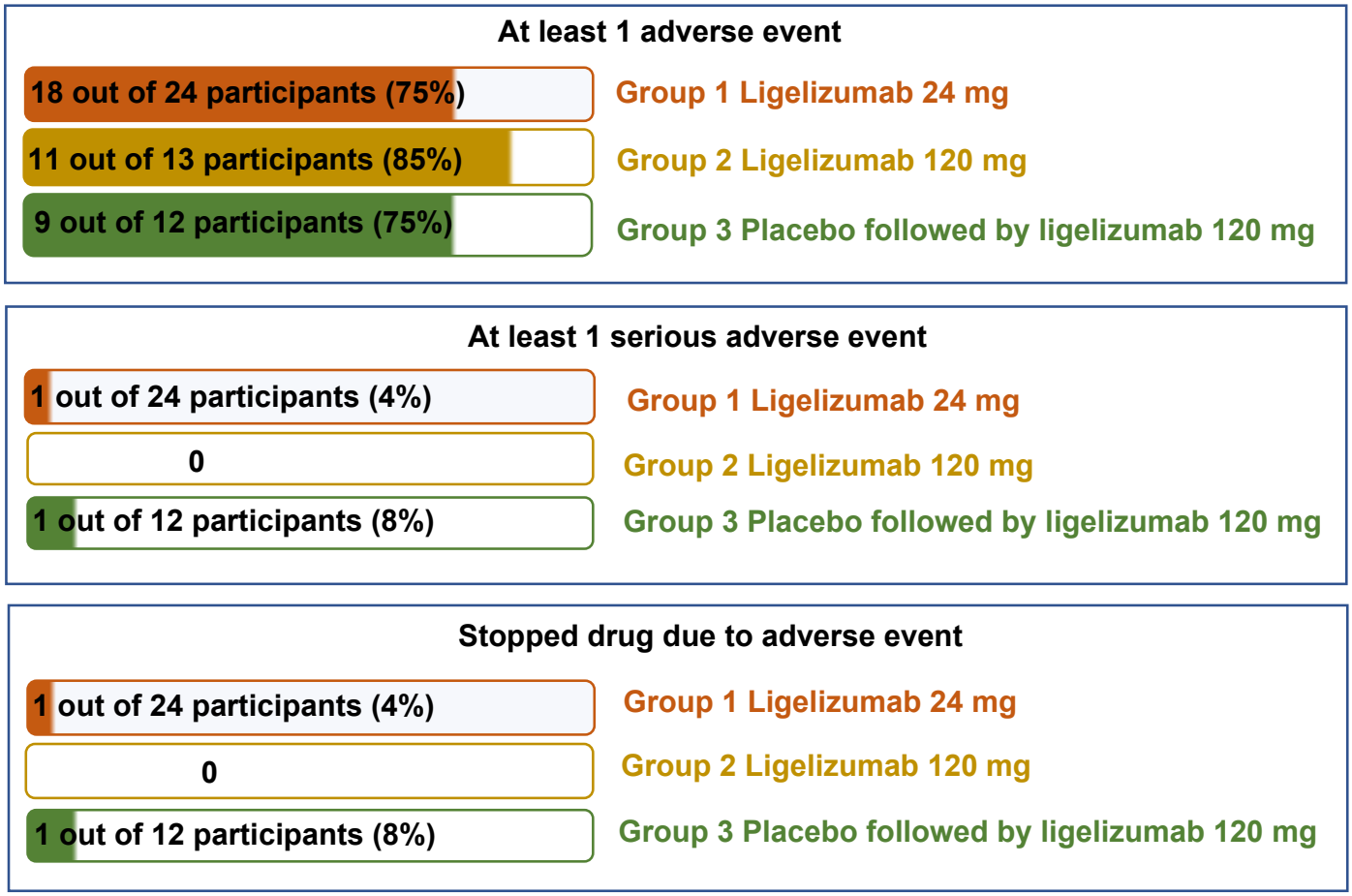
An adverse event is an unwanted sign, symptom, or disease that participants have during a trial

An adverse event is considered "serious" when it is life-threatening, causes lasting problems, or the participant needs hospital care. These problems may or may not be caused by the trial drug

the end of this summary may have more information about all the adverse events that happened in this trial.

How many participants had adverse events?

38 out of 49 (78%) participants had 1 or more adverse events. During the trial, 2 out of 49 (4%) participants stopped the drug early because of adverse events. Serious adverse events happened in 2 out of 49 (4%) participants in the trial.



What were the most common non-serious adverse events?

The most common non-serious adverse events that happened in at least 10% (10 out of 100) of participants in any group are presented below.

Number of Participants (%) With Most Common Non-Serious Adverse Events

Groups (Total number of participants)	Group 1 Ligelizumab 24 mg (24 participants)	Group 2 Ligelizumab 120 mg (13 participants)	Group 3 Placebo followed by ligelizumab 120 mg (12 participants)
Common cold (Upper respiratory tract infection)	3 (13%)	2 (15%)	0
Diarrhea (Diarrhoea)	2 (8%)	0	2 (17%)
Feeling sick to the stomach (Nausea)	1 (4%)	3 (23%)	1 (8%)
Fever (Pyrexia)	2 (8%)	2 (15%)	0
Flu (Influenza)	2 (8%)	1 (8%)	2 (17%)
Headache (Headache)	5 (21%)	1 (8%)	4 (33%)
Infection in the urinary system (Urinary tract infection)	1 (4%)	2 (15%)	1 (8%)
Nose and throat infection (Nasopharyngitis)	7 (29%)	4 (31%)	4 (33%)
Stomach pain (Abdominal pain)	3 (13%)	0	1 (8%)
Stuffy nose (Rhinitis)	0	2 (15%)	0
Vomiting (Vomiting)	1 (4%)	3 (23%)	0
Worsening of CSU (Chronic spontaneous urticaria)	5 (21%)	0	0

What were the serious adverse events?

2 out of 49 (4%) participants

One participant in **Group 1 (Ligelizumab 24 mg)** tried to commit suicide (suicide attempt) but continued with trial treatment and completed the trial.

Another participant in **Group 3 (Placebo followed by ligelizumab 120 mg)** had 2 leaky heart valves (pulmonary valve incompetence and tricuspid valve incompetence) prior to entering the trial. These conditions worsened and required hospitalization and surgery.

How many participants stopped trial drug due to adverse events?

2 out of 49 (4%) participants

One participant in **Group 1 (Ligelizumab 24 mg)** had worsening of CSU.

Another participant in **Group 3 (Placebo followed by ligelizumab 120 mg)** had worsening of 2 leaky heart valves (pulmonary valve incompetence and tricuspid valve incompetence).

How was this trial useful?

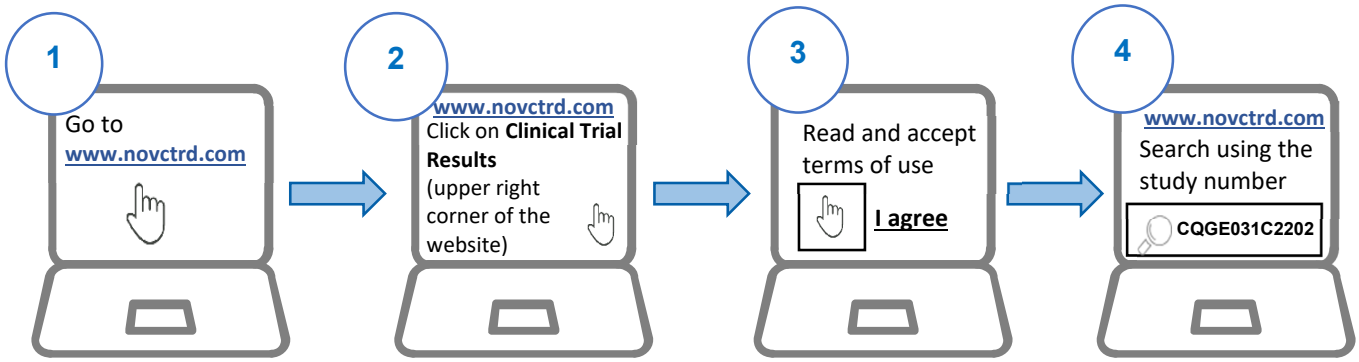
Researchers learned that ligelizumab worked in adolescents between the age of 12 and 18 years with CSU without any safety concerns.

This trial may also help researchers to select a suitable dose of ligelizumab for adolescents between the age of 12 and 18 years with CSU.

Where can I learn more about this trial?

More information about the results and adverse events in this trial can be found in the scientific summary of the results available on the Novartis Clinical Trial Results website (www.novctrd.com).

Please follow the below steps:



You can find more information about this trial on the following websites:

- www.clinicaltrials.gov Use the NCT identifier NCT03437278 in the search field.
- https://www.clinicaltrialsregister.eu/ctr-search/search Use the EudraCT identifier 2017-004207-52 in the search field.

Full clinical trial title: A multicenter, randomized, double-blind, placebo controlled phase 2b dose finding study to investigate the efficacy and safety of ligelizumab (QGE031) in adolescent patients with Chronic Spontaneous Urticaria (CSU)

Trial Dates: The trial started in August 2018 and ended in February 2021.

Thank you

Thank you for taking part in this trial. As a clinical trial participant, you belong to a large community of people around the world. You helped researchers answer important health questions and test new medical treatments.

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Novartis is a global healthcare company based in Switzerland that provides solutions to address the evolving needs of patients worldwide.

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