Clinical Trial Results Summary

A clinical trial to learn about the effects of ligelizumab (QGE031) in Japanese adults with Chronic Spontaneous Urticaria (CSU)

Protocol number: CQGE031C1301

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.

Thanks to the participants for taking part in this trial for the drug ligelizumab, also known as QGE031. They helped researchers learn more about how ligelizumab works in people with CSU.

If the participants have any questions about the trial results, please talk to the doctor or staff at the trial site.

This summary shows the results of a single clinical trial. Other clinical trials may have different findings.

Why was the research needed?

Researchers were looking for a better way to treat Chronic Spontaneous Urticaria (CSU).

Urticaria is a raised, itchy rash that shows up on the skin. It is also called hives, weals, welts, or nettle rash. These rashes may appear on one part of the body and spread across larger areas. An allergic reaction to food, insect stings, or medicines often causes rashes. Normally it goes away quickly, but for some people, the itchy rash comes back with no known cause. An itchy rash that lasts more than 6 weeks and often returns over months or years with no known cause is called CSU. CSU can interfere with sleep and daily activities.

CSU can develop suddenly without an obvious cause. Normally, the immune system makes and uses antibodies (types of proteins in the blood) to identify and fight foreign objects, such as bacteria and viruses. CSU is a type of allergic disease in which the immune system becomes active even when there is no infection. When someone has an allergic reaction, their blood has more of a chemical called histamine. This causes the blood vessels in the affected area of the skin to open up and leak, which often makes the skin red. This extra fluid in the tissues makes the tissues swell and itch. There is also an increase in a blood protein called IgE during allergic reactions. CSU is a type of allergic disease in which IgE and histamine levels in the blood rise for no reason. Antihistamines are medicines that are used to treat CSU. They stop our body from making histamines, which cause allergies

Ligelizumab *(*QGE031) is a trial drug which has not yet been approved for the treatment of CSU. QGE031 attaches itself to IgE so that it is not active anymore. This way, QGE031blocks the effect of IgE and the release of histamine in blood.

Drug	***Pronounced as***
Ligelizumab	li-gu-LIZ-oo-mab

In this trial, researchers wanted to learn about the effects of QGE031 in adult Japanese participants with CSU which could not be controlled with standard treatment (antihistamines).

How long was this trial?

This trial started in April 2019 and ended in January 2022. The entire duration, from enrolling the first participant to the last participant completing the trial was around 2 years and 9 months. An individual participant was in this trial for a maximum of 1 year and 3 months.

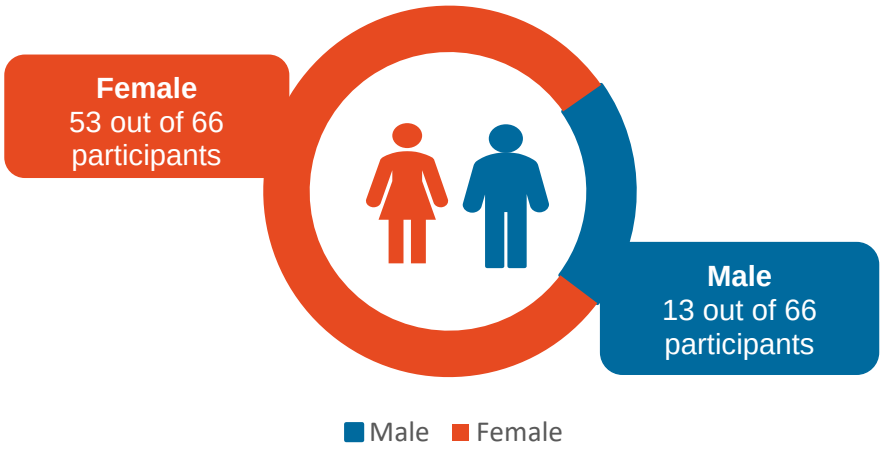
Who was in this trial?

The participants could take part in this trial if they:

- were at least 18 years of age,
- had CSU diagnosed at least for 6 months before the start of the trial,
- had CSU continuously for 6 weeks,
- had CSU which could not be controlled with standard treatment, and
- did not have any other skin disease.

A total of 66 participants from Japan participated in this trial.

Participants' age ranged from 19 to 74 years. 7 participants in the study were over 65 years of age. The remaining 57 participants were between 18 and 65 years. The average age of the participants was 46 years. The majority of participants were female (80%), and all the participants were Japanese (100%).



What treatments did the participants take?

Treatment drug	
	**QGE031** is the trial drug used for the treatment of CSU. It is not an approved drug for CSU. It was given at a dose of 120 milligrams (mg) as an injection under the skin once every 4 weeks.

Along with the treatment above, participants could take other antihistamine treatments to prevent any flareups of CSU.

In this trial, the participants and the clinical trial team knew what treatment each participant took, and all participants took QGE031.

What happened during this trial?

Before treatment

- The trial doctors checked if participants could take part in this trial.

Up to 4 weeks.

During treatment

- All participants received the trial drug QGE031 120 mg as an injection under the skin every 4 weeks until Week 48.
- Participants, trial doctors, and trial staff knew what treatment the participants were receiving.

52 weeks.

After treatment

- No trial drug was given during this period; however, participants were allowed to take medications to relieve their symptoms.
- Researchers closely monitored the overall health of the participants throughout the trial and completed it as planned.

12 weeks.

What were the main results of this trial?

How many participants had medical problems during the trial?

During this trial, 80% of participants (53 out of 66) had at least one adverse event. None of these adverse events were considered serious.

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

An adverse event is any 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.

Number of Participants (%) With Adverse Events

	QGE031 120 mg (Out of 66 participants)
At least 1 adverse event	53 (80%)
At least 1 serious adverse event	11 (17%)
Stopped drug due to adverse event	4 (6%)
Deaths	0

What serious adverse events did the participants have?

None of the participants had any serious adverse events during the trial.

What were the most common non-serious adverse events?

The other adverse events that happened in at least 5% of participants in the study are presented below.

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

	QGE031 120 mg (Out of 66 participants)
Dry itchy skin (Eczema)	9 (14%)
Nose and throat infection (Nasopharyngitis)	8 (12%)
Fever (Pyrexia)	6 (9%)
Back pain	5 (8%)
Skin rash (Dermatitis contact)	5 (8%)
Headache	4 (6%)
Tooth decay (Dental caries)	4 (6%)
Worsening of hives (Urticaria)	4 (6%)

How many participants stopped trial drug due to adverse events?

During the trial, 4 out of 66 participants (6%) stopped the trial drug due to adverse events. The adverse events leading to treatment discontinuation were worsening of hives (urticaria), raised red bumps on the skin (rash maculo-papular), and abnormal liver function (hepatic function abnormal).

What were the other results of this trial?

Did the participants show a decrease in their itch and hives during 52 weeks of treatment compared to the start of the trial?

Participants showed steady decrease in both their itch and hives during the 52 weeks of treatment compared to the start of the trial.

How many participants' itch and hives completely disappeared at Week 52?

There was a steady increase in the number of participants whose itch and hives disappeared during the study. Itch and hives symptoms completely disappeared at Week 52 in 50% of participants (28 out of 56).

How many participants reported their CSU symptoms as having no impact on their quality of life at Week 52?

Sixty-nine percent (69%) of participants, or 44 out of 64 participants, reported their CSU symptoms had no impact on their quality of life at Week 52.

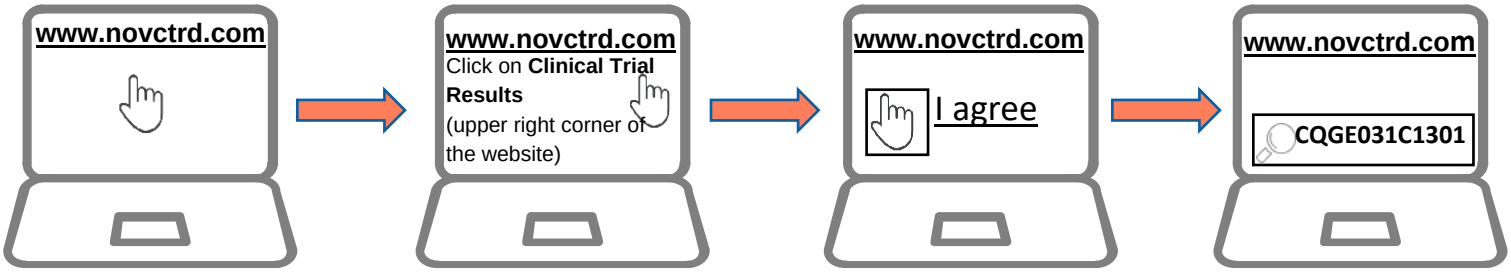
How was this trial useful?

The trial helped researchers learn about the effects of QGE031 in treating Japanese adults with CSU, which could not be controlled with antihistamines. QGE031 helped improve the disease condition and the quality of life without any new safety issues.

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 website:

- **www.clinicaltrials.gov** Use the NCT identifier NCT03907878 in the search field.

Full clinical trial title: A multi-center, open-label study to investigate the safety/tolerability and efficacy of ligelizumab (QGE031) in the treatment of adult Japanese patients with Chronic Spontaneous Urticaria (CSU) inadequately controlled with H1 antihistamines

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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