NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn about the long-term safety of CFZ533 as a pre-filled syringe in people with Sjögren's syndrome

Thank you!

Thank you to the participants who took part in the clinical trial for **Sjögren's syndrome (SjS)**. Every participant helped the researchers learn more about the trial drug **CFZ533**, also called iscalimab.

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. We hope this helps the participants understand their important role in medical research.

Trial information

Trial number: CCFZ533B2201E1

Novartis drug studied: **CFZ533**, also called iscalimab

Sponsor: Novartis

If you were a participant and have any questions about the results, please talk to the doctor or staff at the trial site.

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

What was the main purpose of this trial?

The purpose of this trial was to learn about the long-term safety of **CFZ533** as a pre-filled syringe in people with Sjögren's syndrome (SjS).

This trial was an extension trial. In an **extension trial**, researchers invite participants from another trial of the same trial drug to join. The other trial is called the **core trial**. The extension trial may allow participants to continue to take the trial drug and allow researchers to continue to learn about the trial drug's safety and effects after the core trial ends. The core trial's number was CCFZ533B2201.

Sjögren's syndrome (SjS) also called Sjögren's disease, is an **autoimmune disease** in which the body attacks and damages the glands that make fluid, like tears and saliva.

Common symptoms of SjS include:

- Dry eyes and mouth
- Pain
- Fatigue

SjS can also damage other parts of the body. This causes problems and symptoms throughout the body, such as the joints, kidneys, muscles, skin, lungs, nervous system, and blood. Women are more likely to have SjS than men.

CFZ533, also called iscalimab, is a trial drug designed to block a protein in the body that is part of the immune system. By blocking this protein, **CFZ533** may help prevent the immune system from attacking healthy cells.

A **pre-filled syringe** means the correct amount of **CFZ533** was already inside the syringe. In this trial, participants gave themselves injections of **CFZ533** at home using the pre-filled syringe. In the core trial, CCFZ533B2201, trial staff gave participants the injections of **CFZ533**.

Trial drug

CFZ533 also called iscalimab

Pronounced as

is-cal-i-mab

The trial's purpose was to answer this main question:

- What medical problems, also called adverse events, happened during this trial?
  - ↳ An **adverse event** is any sign or symptom that participants have during a trial. Adverse events **may** or **may not** be caused by treatments in the trial.

How long was this trial?

The trial began in January 2021 and ended in August 2024. Each participant was in the trial for up to 14 months. The participants started this trial on different dates.

Who was in this trial?

206 participants with SjS joined this trial – 5 men and 201 women. Participants' ages ranged from 23 to 80 years. Their average age was 52 years.

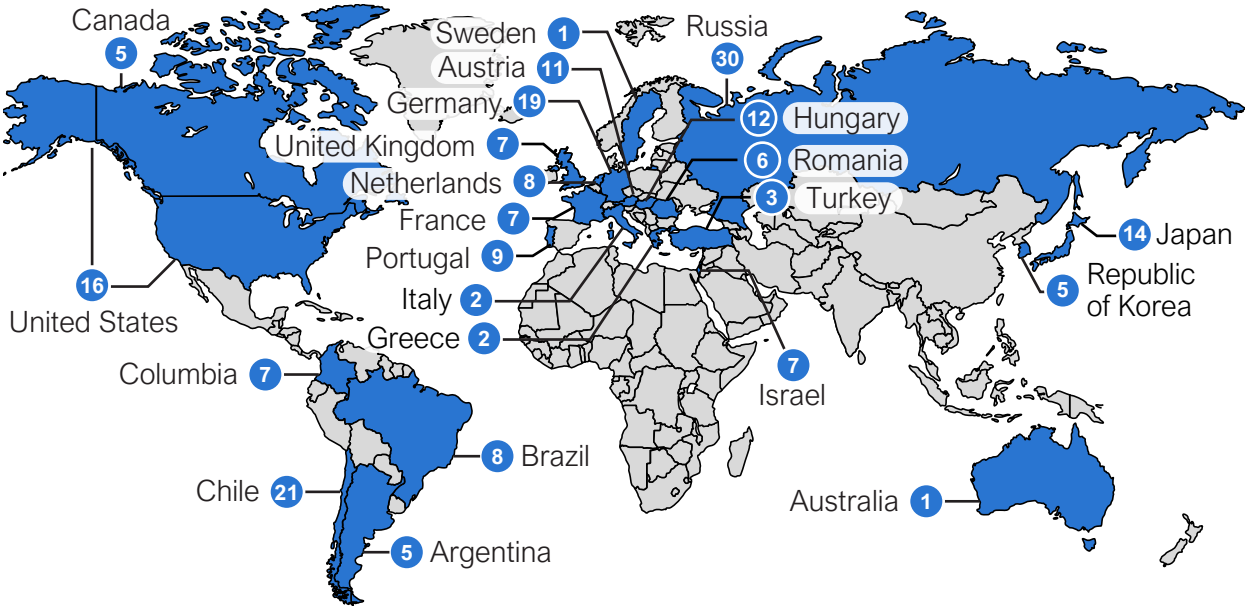
The number of participants by race is shown below.

Race

5 American Indian or Alaska Native	22 Asian
7 Black or African American	170 White
2 Unknown	

The participants could take part in this trial if they completed the core trial, CCFZ533B2201.

206 participants from 23 countries received treatment. The map below shows the number of participants who took part in each country.



What treatments did the participants receive?

The treatment in this trial was:

CFZ533, which was received as injections under the skin using a pre-filled syringe every 1 or 2 weeks. This trial looked at 2 doses:

- **300 milligrams (mg) CFZ533**
- **600 mg CFZ533**

Along with the treatment above, participants could take other treatments for SjS.

Researchers assigned participants to receive either 300 mg or 600 mg of **CFZ533** based on:

- The dose they received during the core trial, and
- Whether their SjS symptoms became less severe during the core trial

At the start of this extension trial, the participants, researchers, and trial staff did not know what dose of **CFZ533** the participants were receiving. Some trials are done this way because knowing what treatment the participants receive can affect the results of the trial. Doing a trial this way helps to make sure that the results are looked at with fairness across all treatments.

To make sure participants didn't know what dose they were receiving, each participant received their assigned treatment as 2 injections. Participants who were assigned:

- **600 mg CFZ533** received 2 injections of **CFZ533**
- **300 mg CFZ533** received 1 injection of **CFZ533** and 1 injection of **placebo**

When the core trial ended, participants who joined this extension trial learned what dose they were receiving in this trial. Participants who were receiving **300 mg CFZ533** and **placebo**, stopped receiving the **placebo**, and continued only receiving **CFZ533**.

What is a placebo?

Placebo looks like the trial drug but does not have any drug in it.

What happened during this trial?

Before treatment

The trial staff invited participants who were in the core trial, CCFZ533B2201, to join this extension trial.

The trial staff checked to make sure the participants could be in this trial.

During treatment

Up to 1 year

206 participants received one of these treatments:

- **600 mg CFZ533**: 152 participants
- **300 mg CFZ533**: 54 participants

All participants received their first 3 doses once a week, and then once every 2 weeks after that.

After treatment

14 weeks

Trial staff checked participants for any medical problems for up to 14 weeks after their last dose of trial treatment.

Trial staff checked the participants' general health throughout the trial.

What were the main results of this trial?

What medical problems, also called adverse events, happened during this trial?

Trial doctors keep track of all medical problems, also called **adverse events**, that happen in trials. They track adverse events even if they think the adverse events are not related to the trial treatments.

Many trials are needed to know if a drug or treatment causes an adverse event.

This section is a summary of the adverse events that happened from the start of trial treatment until 14 weeks after the last dose of trial treatment.

An **adverse event** is:

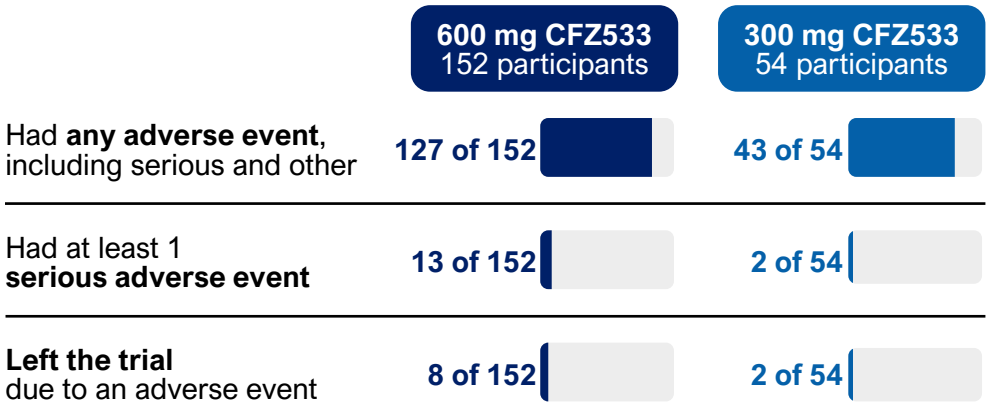
- Any **sign or symptom** that the participants have during a trial
- Considered **serious** when it is life-threatening, causes lasting problems, the participant needs hospital care, or results in death

Adverse events **may** or **may not** be caused by treatments in the trial.

A total of 170 of 206 participants had adverse events, including serious and other adverse events. 15 participants had adverse events that were considered serious. 10 participants left the trial due to an adverse event. No participants died.

The researchers concluded there were no new safety concerns for **CFZ533** in this trial, compared to past trials.

How many participants had adverse events?



What serious adverse events did the participants have?

15 participants had serious adverse events. No participants died.

The most common serious adverse event that happened was:

- **Fever and low number of a type of white blood cell called neutrophils** (febrile neutropenia)

This serious adverse event happened in 2 participants who received **600 mg CFZ533**.

Additional serious adverse events happened in fewer participants.

What other (not including serious) adverse events did the participants have?

The table below shows the most common other adverse events that happened. Additional adverse events happened in fewer participants.

	600 mg CFZ533 152 participants	300 mg CFZ533 54 participants
COVID-19	39 of 152 26%	16 of 54 30%
Common cold Nasopharyngitis	22 of 152 14%	9 of 54 17%
Infection in the nose, throat, and airways Upper respiratory tract infection	19 of 152 13%	5 of 54 9%
UTI Urinary tract infection	16 of 152 11%	5 of 54 9%
Flu Influenza	7 of 152 5%	7 of 54 13%

What was learned from this trial?

Researchers learned about the long-term safety of **CFZ533** as a pre-filled syringe in people with Sjögren's syndrome (SjS).

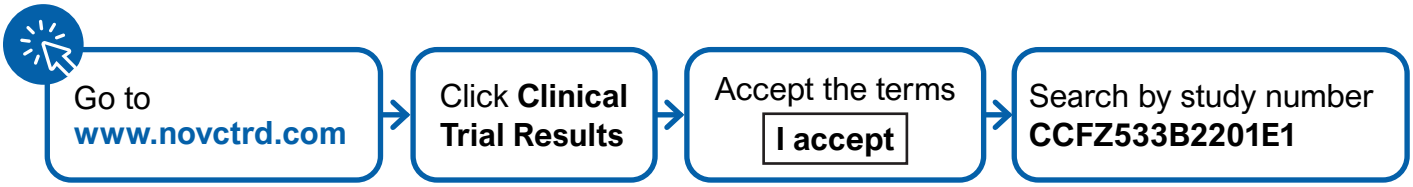
The researchers concluded that there were no new safety concerns for **CFZ533** in this trial, compared to past trials.

When this summary was written, the sponsor had no plans for future trials of **CFZ533** in people with SjS.

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**

Follow these steps to find the scientific summary:



For more information about this trial, go to any of these websites:

- **clinicaltrials.gov** – search using the number **NCT04541589**
- **clinicaltrialsregister.eu** – search using the number **2020-001942-20**

Other trials of **CFZ533**, including the core trial, may appear on the public websites above. When there, search for **CFZ533** or iscalimab, or the core trial study number CCFZ533B2201.

Full clinical trial title: A TWINSS Extension trial to evaluate the safety and tolerability of CFZ533 (iscalimab) at two dose levels administered subcutaneously in patients with Sjögren's Syndrome

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