NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn more about the effects of canakinumab in Japanese people with Adult-Onset Still's Disease

Thank you!

Thank you to the participants who took part in the clinical trial for **Adult-Onset Still's Disease (AOSD)**. Every participant helped learn more about the trial drug **canakinumab**, also called **ACZ885**.

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: CACZ885G1302

Novartis drug studied: **canakinumab,** also called **ACZ885**.

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 findings.

What was the main purpose of this trial?

The purpose of this trial was to learn about the effects of **canakinumab** in Japanese people with **Adult-Onset Still's Disease (AOSD)**.

AOSD is a rare inflammatory condition. Symptoms include:

High fevers **Sore throat** **Skin rash** **Painful joints (arthritis)**

Problems with organs like the liver and spleen

The cause of **AOSD** is not known. Activation of the immune system, the body's natural defense system, and increased amounts of several proteins that are part of the immune system, such as interleukin-1 (IL-1), are thought to be involved. This has led to different drugs being tested to find treatments that may help people with **AOSD**. Standard treatment consists of medicines to reduce inflammation and often includes corticosteroids.

Canakinumab is a type of drug known as a monoclonal antibody that binds and blocks a protein called IL-1β. It is approved in several countries for the treatment of a similar condition called systemic juvenile idiopathic arthritis as well as other inflammatory conditions, and for **AOSD** outside of Japan. The aim of this study is to learn about **canakinumab** for Japanese people with **AOSD** and to gain approval for its use in Japan.

What are monoclonal antibodies?

Antibodies are made by our immune system to fight off infections or anything that is harmful to our body. Monoclonal antibodies are a type of antibody that is made in a lab and are developed to target specific proteins in the body.

The trial's purpose was to answer these main questions:

- Did participants' symptoms of **AOSD** improve after 8 weeks of treatment?
- Were participants able to reduce their use of corticosteroids after 28 weeks of treatment?
- 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 March 2021 and ended in April 2025. Participants could continue receiving **canakinumab** for as long as they were benefiting up to the time when **canakinumab** gained approval for the treatment of **AOSD** in Japan or the trial ended.

Canakinumab received approval for **AOSD** in Japan in March 2025.

At the end of this trial, the sponsor, Novartis, created a report of the trial results. This summary is based on that report.

Who was in this trial?

14 participants with **AOSD** in Japan received treatment in this trial – 4 males and 10 females. Their average age was 51 years.

All participants were Asian by race.

Participants **could take part** in this trial if they:

- Were aged over 16 years old with a confirmed diagnosis of **AOSD**
- Had active disease at time of trial start
- Had a history of inadequate response to corticosteroids

What treatments did the participants receive?

The treatment in this trial was:

Canakinumab: given as an injection under the skin (subcutaneous injection), at a dose of 4 milligrams per kilogram (mg/kg) of body weight, every 4 weeks. Any participant who needed a single dose over 150 mg received 2 injections.

The participants, researchers, and trial staff knew that all participants received **canakinumab**.

What happened during this trial?

Before treatment Up to 1 month

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

During treatment For as long as participants were benefiting

All 14 participants received **canakinumab** at a dose of 4 milligram per kilogram (mg/kg) of body weight, up to a maximum of 300 mg, every 4 weeks throughout the trial.

After treatment Up to 1 month

Trial staff checked participants' general health and for any medical problems for up to 1 month after their last dose of trial treatment.

Trial staff checked participants' for their overall health throughout the trial.

What were the main results of this trial?

Did participants' symptoms of AOSD improve after 8 weeks of treatment?

After 8 weeks of treatment, half of the participants **(7 out of 14)** had an improvement in their **AOSD** symptoms.

To learn if participants' symptoms improved researchers used a tool called the **adapted ACR 30 response**. To reach an **adapted ACR 30 response** participants need to have a reduction of 30% or more in at least 3 markers included in the tool and that they did not have a fever in the week before assessment. A participant's response was measured by calculating the percentage improvement from before treatment to week 8.

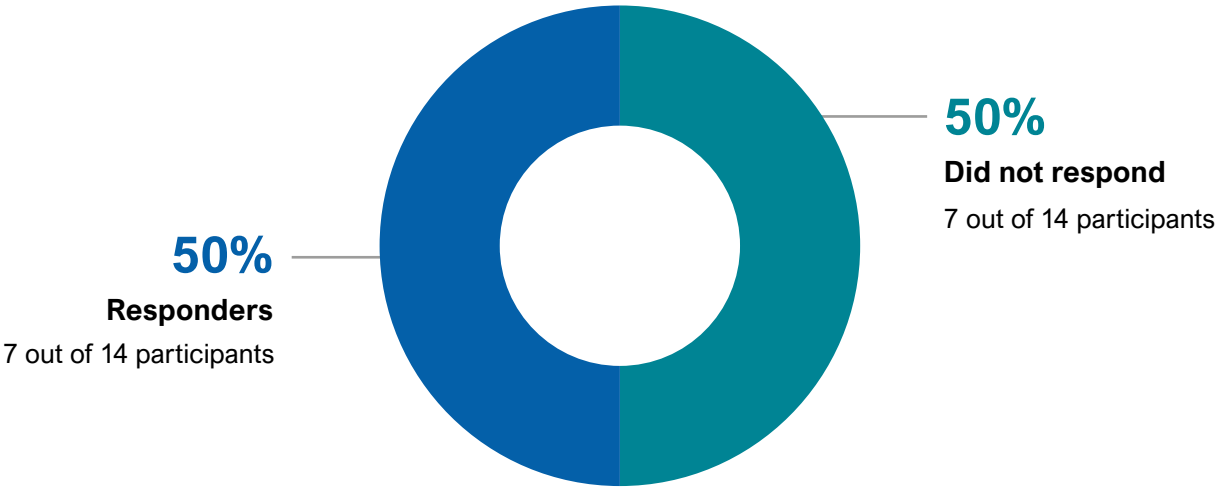
What is the adapted ACR 30 tool?

The adapted American College of Rheumatology 30 (ACR 30) tool measures several markers of **AOSD** including:

- disease activity
- a person's ability to carry out daily activities
- the number of joints with pain and swelling
- a measure of inflammation from a blood sample
- whether a person has a fever

Adapted ACR 30 response after 8 weeks of treatment

Out of 14 participants, 7 achieved the response and 7 did not (50% each).



What were the other results of this trial?

Were participants able to reduce their use of corticosteroids after 28 weeks of treatment?

After 28 weeks of treatment, **7 out of 14 participants** were able to reduce their dose of corticosteroids.

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 do this even if they think the adverse events are not related to the trial treatments.

Researchers need results from many trials to decide if a drug or treatment causes an adverse event.

This section is a summary of the adverse events that happened from the day participants started the treatment until 1 month after the last 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.

All participants, **14 out of 14**, had adverse events, including serious and other adverse events.

- 6 participants had serious adverse events
- No participants died

The researchers concluded there were no unexpected safety concerns for **canakinumab** in this trial.

What serious adverse events did the participants have?

6 participants had serious adverse events.

These serious adverse events happened in 1 participant each, with some participants having more than one:

- **Inflammation of the gut** (enteritis)
- **Bleeding in the gut** (intestinal haemorrhage)
- **Liver not working as normal** (hepatic function abnormal)
- **COVID-19**
- **COVID 19 lung infection** (COVID-19 pneumonia)
- **Skin infection** (cellulitis)
- **Bone infection** (osteomyelitis)
- **Lung infection** (pneumonia)
- **Narrowing of the spinal cord in the lower back** (lumbar spinal stenosis)
- **Rash**

The following serious adverse event happened in 3 participants:

- **Worsening of AOSD** (Still's disease)

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

The table below shows the most common other adverse events.

	Canakinumab 14 participants
Common cold Nasopharyngitis	**6 of 14 (43%)**
Tooth decay Dental caries	**4 of 14 (29%)**
Nausea	**4 of 14 (29%)**
Dry eye	**3 of 14 (21%)**
Diarrhea	**3 of 14 (21%)**
Swelling in the hands, feet, arms or legs Oedema peripheral	**3 of 14 (21%)**
Pink eye Conjunctivitis	**3 of 14 (21%)**
Herpes virus infection Herpes Zoster	**3 of 14 (21%)**
Worsening of AOSD Still's disease	**3 of 14 (21%)**
Headache	**3 of 14 (21%)**
Itching Pruritus	**3 of 14 (21%)**
Rash	**3 of 14 (21%)**
Hives Urticaria	**3 of 14 (21%)**

What was learned from this trial?

Researchers learned about the effects of **canakinumab** in people with **Adult-Onset Still's Disease (AOSD)**.

The researchers concluded that for participants who received **canakinumab**:

- Half had an improvement in their symptoms of **ASOD**, achieving an adapted ACR 30 response after 8 weeks of treatment
- 7 out of 14 participants were able to reduce their dose of corticosteroids after 28 weeks of treatment.
- There were no unexpected safety concerns for **canakinumab** in this trial.

During the trial, canakinumab received approval for the treatment of **Adult-Onset Still's Disease** in Japan. When this summary was written, the sponsor had no plans for future trials of **canakinumab** in people with **Adult-Onset Still's Disease**.

Where can I learn more about this trial?

To learn more about the results and adverse events in this trial, read the scientific summary of the results. It is available on the Novartis Clinical Trial Results website **www.novctrd.com**

Follow these steps to find the scientific summary:

Go to **www.novctrd.com** → Click **Clinical Trial Results** → Accept the terms ☑ **I accept** → Search for **CACZ885G1302**

For more information about this trial go to this website:

clinicaltrials.gov – search using the number **NCT04717635**

Other trials of **canakinumab** may appear on the public websites above. When there, search for **canakinumab** or **ACZ885**.

Full clinical trial title: An open-label, single-arm, active-treatment study to evaluate efficacy and safety of canakinumab (ACZ885) administered for at least 48 weeks in Japanese patients with Adult-Onset Still's Disease (AOSD).

NOVARTIS

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

1-888-669-6682 (US); +41-61-324 1111 (EU)

www.novartis.com/clinicaltrials