NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn more about the effects and safety of DFV890 in people with familial cold auto-inflammatory syndrome (FCAS)

Thank you!

Thank you to the participants who took part in the clinical trial for FCAS. Every participant helped the researchers learn more about the trial drug **DFV890**.

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: CDFV890A12201
Drug studied: DFV890
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 and safety of DFV890 in people with familial cold auto-inflammatory syndrome, or FCAS.

FCAS is a rare disease where the immune system overreacts to cold temperatures.

The **immune system** is made up of cells and proteins that help the body fight off infections or other possible harms.

When people with FCAS are in cold temperatures, they have symptoms like fever, rash, joint and muscle pain, and pink eye. Some people with FCAS also get headaches and feel tired or lack energy, even when not in the cold.

What does auto-inflammatory mean?

Auto-inflammatory means that the white blood cells from the **immune system** attack the body's own healthy tissues by mistake.

DFV890 is a trial drug designed to block a certain part of the immune system. Researchers think blocking this part may stop the immune system from overreacting to cold temperatures.

The trial purpose was to answer these main questions:

- Did participants' immune systems react less when being in cold temperatures after taking DFV890?
- What adverse events did the participants have during this trial?
  - ↳ An **adverse event** is any sign or symptom that the participants have during a trial.

How long was this trial?

The trial began in September 2021 and ended in May 2023.

Who was in this trial?

4 participants with FCAS received treatment in this trial – 3 men and 1 woman. Participants' ages ranged from 28 to 62 years. Their average age was 41 years. The number of participants by race are shown below.

Race

3 White	1 Other

The participants could take part in this trial if they:

- Stopped taking certain medicines for an overactive immune system before joining the trial
- Did not have health conditions that lowered their immune system

4 participants from 3 countries received treatment. The trial took place in these countries:

- France | 2 participants
- Germany | 1 participant
- United States | 1 participant

What treatment did the participants receive?

The treatment in this trial was:

DFV890: Participants took 100 milligrams (mg) by mouth as pills twice a day.

Along with the treatment above, participants could take other medicines for FCAS, such as the pain relievers acetaminophen and ibuprofen.

In this trial, the participants, researchers, and trial staff knew what treatment each participant took. All participants took DFV890.

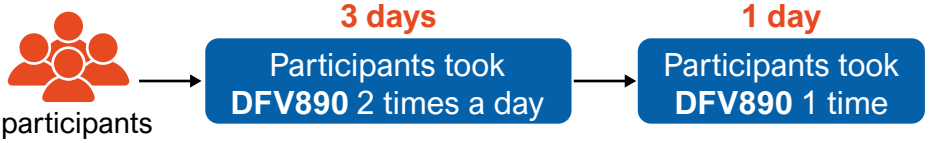
What happened during this trial?

Before treatment — **Up to 1 year**

Trial staff checked the participants' FCAS symptoms and general health to make sure they could be in this clinical trial.

During treatment — **4 days**

4 participants took DFV890 by mouth as pills twice a day for 3 days. On the morning of the 4th day, participants took DFV890 one time.



Trial staff checked the participants' FCAS symptoms and general health.

After treatment — **About 1 month**

After treatment, participants returned to their trial site 1 time to check on their FCAS symptoms and general health. Trial staff also called each participant about 30 days after treatment to check on their health.

What were the main results of this trial?

Did participants' immune systems react less when being in cold temperatures after taking DFV890?

There were too few participants for researchers to conclude if participants' immune systems reacted less to cold temperatures after taking DFV890.

During the trial, the participants had a **cold challenge** 2 times:

- 1 time before treatment
- 1 time after taking DFV890 for 4 days

For each cold challenge, the participants stayed in a cold room for 45 minutes while wearing light clothing, such as a t-shirt and shorts. The cold room was about 4°C, or about 39°F.

The researchers measured the number of **white blood cells** (WBCs) in the participants' blood samples before and after each cold challenge. Then, they compared the change in the number of white blood cells between the 2 challenges.

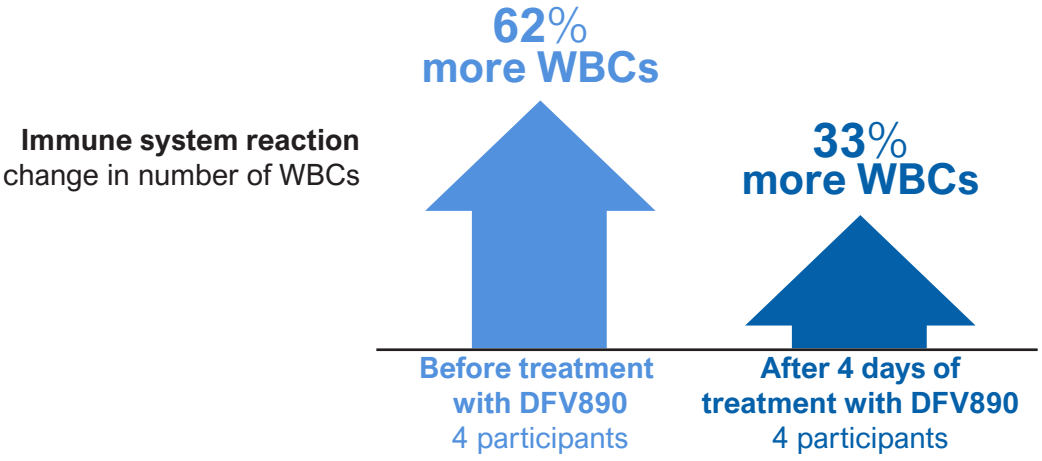
They found that after the second cold challenge, the white blood cells went up less after the participants took DFV890. However, the researchers concluded there were too few participants to know if this difference was meaningful.

What are white blood cells?
White blood cells (WBCs) are part of the immune system and help the body fight infections or other possible harms.

In FCAS, the immune system overreacts to cold temperatures by making too many WBCs.

Change in how participants' immune systems reacted to the cold

The graph below shows how much the number of participants' white blood cells (WBC) went up after each cold challenge.



A **lower** percent of WBCs means the immune system reacted **less** to the cold.

What were the other results of this trial?

Did participants have less severe FCAS symptoms after they took DFV890?

Overall, participants' FCAS symptoms were less severe after they took DFV890. However, there were too few participants for researchers to conclude if the change was meaningful.

To learn this, the trial doctors and participants rated the participants' FCAS symptoms using questionnaires before and after the 2 cold challenges.

What adverse events did the participants have?

Trial doctors keep track of all **adverse events** that happen in trials, 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 a participant's first dose of trial treatment until 30 days after their last dose or their last study visit.

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 4 participants had at least one adverse event during the trial. None of the participants had adverse events which were considered serious. None of the participants left the trial because of an adverse event. The researchers concluded there were no new safety concerns for DFV890 in this trial.

How many participants had adverse events?

Participants who:	DFV890 4 participants
Had at least 1 **serious adverse event**	**0 of 4** 0%
Had at least 1 **other adverse event**	**4 of 4** 100%
Left the trial due to an adverse event	**0 of 4** 0%
Died during the trial	**0 of 4** 0%

What serious adverse events did the participants have?

None of the participants had serious adverse events, including no deaths.

What other adverse events did the participants have?

All 4 participants had other adverse events.

The table below shows the other adverse events that happened in **2 or more** participants across the treatment and follow-up periods. Additional adverse events happened in fewer participants.

	DFV890 4 participants
Joint pain Arthralgia	**3 of 4** 75%
Rash after exposure to cold Cold urticaria	**2 of 4** 50%
Feeling tired or lacking energy Fatigue	**2 of 4** 50%
Rash Urticaria	**2 of 4** 50%

What was learned from this trial?

Researchers learned about the effects and safety of DFV890 in people with FCAS.

Because there were too few participants, the researchers could not conclude if:

- The participants' immune system reacted less after taking DFV890
- Their FCAS symptoms were less severe after taking DFV890

The researchers concluded there were no new safety concerns for DFV890 in this trial.

Researchers look at the results of many trials to learn about the effects and safety of new treatments. There are no other trials planned for DFV890 in people with FCAS.

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:

Go to **www.novctrd.com** → Click **Clinical Trial Results** → Agree to the terms ☑ **I agree** → Search for **CDFV890A12201**

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

- **clinicaltrials.gov** – search using the number **NCT04868968**
- **clinicaltrialsregister.eu/ctr-search/search** – search using the number **2020-005948-33**

Full clinical trial title: An open-label, single arm phase II study of DFV890 to assess the safety, tolerability and efficacy in participants with familial cold auto-inflammatory syndrome (FCAS)

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