NOVARTIS

Clinical Trial Results Summary

A Study to Learn About the Effects of CSJ117 in People With Severe Uncontrolled Asthma

Thank you!

Thank you to the participants who took part in the clinical trial for severe uncontrolled asthma. Every participant helped the researchers learn more about **CSJ117**.

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 summary helps the participants to understand their important role in medical research.

Trial information

Trial number: CCSJ117A12201C
Drug studied: CSJ117
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.

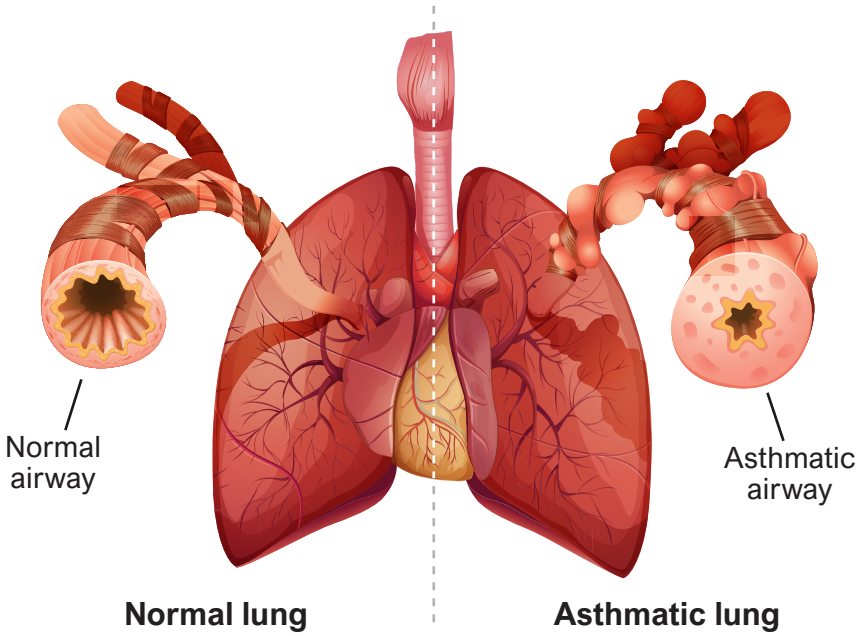
What was the main purpose of this trial?

Asthma is a long-term condition that affects the lungs. It causes the **airways to swell up**, become narrower, and fill up with mucus. This means that less oxygen can travel into the lungs, making it harder for a person to breathe. Common symptoms of asthma are:

- Coughing
- Wheezing
- Shortness of breath
- Chest tightness

For some people, asthma can be caused by breathing in certain substances such as pollen, dust, and smoke. This type of asthma is called allergic asthma.

People with asthma often have asthma attacks. During an asthma attack, the airways become swollen and inflamed, releasing extra mucus. If left untreated, asthma can lead to serious problems.



There is no cure for asthma. Current treatments help to reduce the symptoms through opening the airways and making breathing easier. People with asthma are usually given medicines using an inhaler, which delivers the medicines directly to the lungs. The common treatments used are:

- **Inhaled corticosteroids (ICS),** which reduce inflammation
- **Long-acting beta-agonists (LABA),** which open the airways and help in breathing

Sometimes, these treatments may not work for all patients or may stop working for some patients. As a result, there is a need to find new treatments.

A protein called thymic stromal lymphopoietin (TSLP) is thought to cause asthma symptoms. High amounts of TSLP are usually found in the lungs of asthma patients. The trial drug, **CSJ117**, is designed to block TSLP, which may reduce asthma symptoms. CSJ117 is not currently approved to treat asthma.

In this trial, researchers wanted to find out if CSJ117 can reduce asthma symptoms and improve lung function in participants with severe uncontrolled asthma.

The main questions that researchers wanted to answer were:

- How did participants' lung function change after treatment at Weeks 8 and 12?
- What adverse events did participants have during this trial?
  - ↳ An **adverse event** is any sign or symptom that participants have during a trial.

How long was this trial?

The trial began in September 2020 and ended in September 2022. The entire duration was about 2 years. The trial was designed so that each participant would take part for up to 34 weeks.

Participants who completed 12 weeks of treatment were given the option to continue treatment in a separate extension trial (CCSJ117A12201E1). Both this trial and the extension trial ended earlier than planned due to the sponsor's decision. The decision was not due to any safety concerns with CSJ117.

When the trial ended, researchers created a report of the trial results. This summary is based on that report.

Who was in this trial?

335 participants with severe uncontrolled asthma received treatment in this trial. Participants' ages ranged from 19 to 75 years. Their average age was 51 years.

The number of participants by gender and race are shown below.

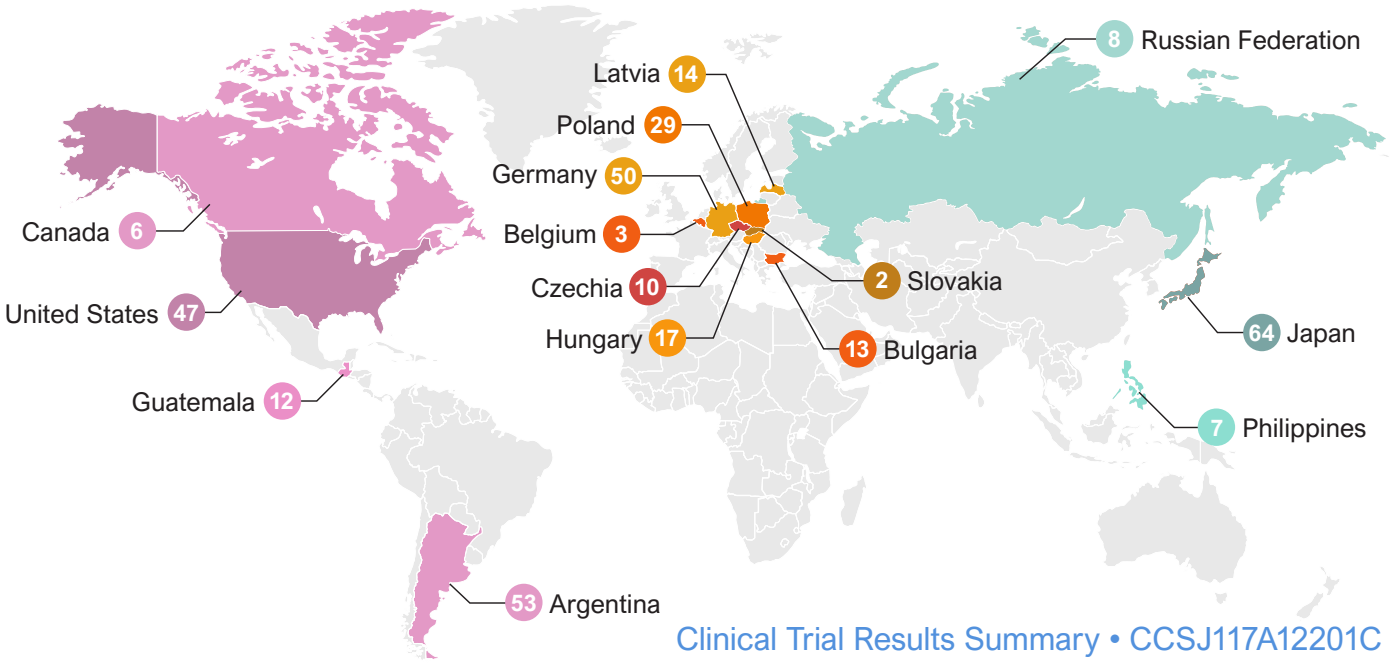
Gender

- 126 Men
- 209 Women

Race

- 73 Asian
- 9 American Indian or Alaska Native
- 10 Black or African American
- 243 White

335 participants from **15 countries** received treatment. The map below shows the number of participants who took part in each country.



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

- Had a diagnosis of asthma at least 1 year before their first trial visit.
- Were receiving medium or high dose ICS plus LABA.

Participants **could not take part** in this trial if they:

- Were currently smokers or had a long history of smoking.

What treatments did the participants receive?

Researchers studied the following treatments:

CSJ117: Participants received one of the following doses: **0.5, 1, 2, 4, or 8 milligrams (mg).** They were provided as capsules, taken through an inhaler once a day. An inhaler is a device that is used to deliver medicines directly into the lungs by breathing.

Placebo: Looks like the trial drug but does not have any active drug in it. Using a placebo helps researchers better understand the effect of the trial drug.

Participants would continue to take their regular asthma medicines, ICS and LABA, along with the trial drugs. A rescue medication was provided to participants to take in case immediate relief was needed.

In this trial, the participants, trial doctors, and trial staff did not know which doses of CSJ117 or placebo that participants received. Doing a trial this way helps to make sure that the results are looked at with fairness across all treatments.

What happened during this trial?

Before treatment

Up to 2 weeks

At the start of the trial, all participants were checked to make sure they could take part.

This included a physical exam, tests for lung function, and blood and urine tests. Researchers asked questions about participants' medical history.

Participants had their levels of blood eosinophils checked. Eosinophils are a type of white blood cell that are involved in allergies.

Participants were given an electronic diary to record aspects of their asthma and lung function. Participants entered their daily asthma symptoms and any use of rescue medications.

Placebo Period

Up to 4 or 8 weeks

Before treatment with CSJ117 began, all participants took part in an initial period where they inhaled placebo for 4 weeks. This was done to check if the participants took the medications properly and completed their diaries regularly.

If a participant experienced a worsening of their asthma or an infection in their airways, they were closely observed and stayed in the Placebo Period for up to 8 weeks.

During treatment

Up to 12 weeks (Week 1 to Week 12)

After the Placebo Period, all participants received **CSJ117** or **placebo** for 12 weeks. Participants were randomly assigned to 1 of 6 treatment groups by a computer.

The treatment groups were:

- **CSJ117, 0.5 mg:** 36 participants
- **CSJ117, 1 mg:** 37 participants
- **CSJ117, 2 mg:** 37 participants
- **CSJ117, 4 mg:** 76 participants
- **CSJ117, 8 mg:** 74 participants
- **Placebo:** 75 participants

Throughout the Treatment Period, researchers:

- Took blood samples and performed lung function tests
- Checked participants' electronic diaries
- Checked participants for any new or worsening medical problems

After treatment

Up to 12 weeks

Participants remained in the trial for an additional 12 weeks after their last dose of trial drug. Researchers checked participants' lung function and recorded medical problems. Participants also had multiple blood tests.

What were the main results of this trial?

How did participants' lung function change after treatment at Weeks 8 and 12?

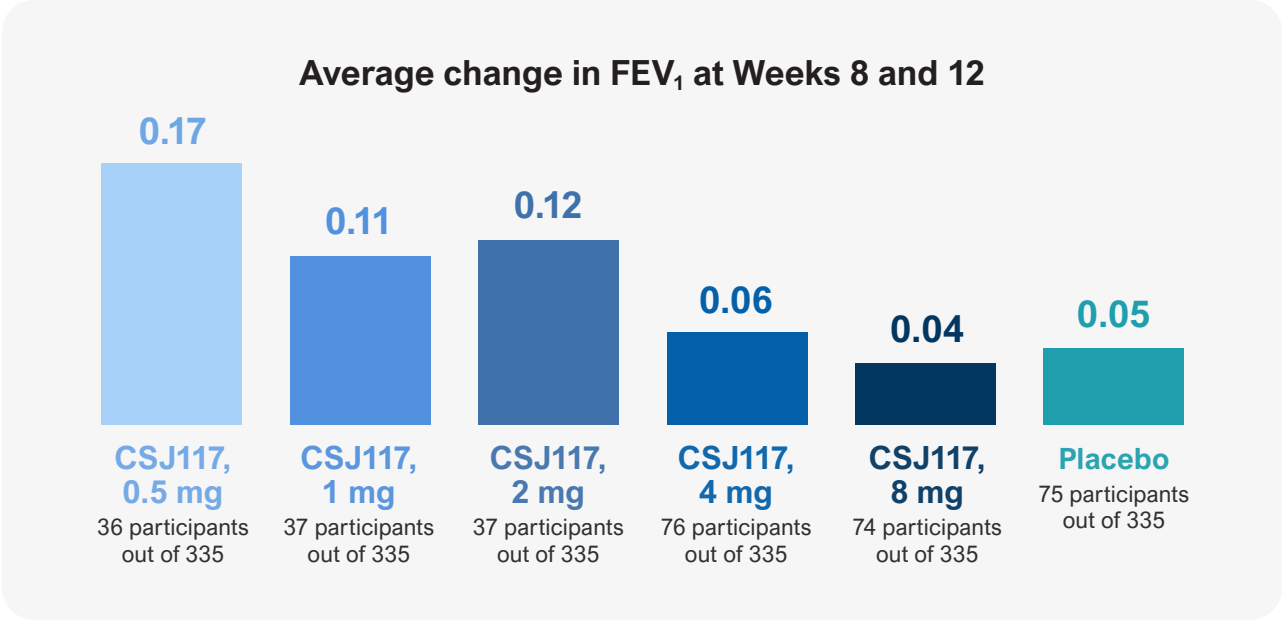
FEV_1 went up the most in the **CSJ117, 0.5 mg treatment group,** when compared to the **placebo** group.

Researchers found that this difference from the **placebo** group was not big enough to be meaningful.

Researchers wanted to know how participants' lung function changed after treatment. They tested lung function by measuring the **forced expiratory volume in one second (FEV_1).** FEV_1 is how much air a participant could blow out (exhale) with force in one second. FEV_1 is measured in liters (L).

FEV_1 was measured before the trial drug was given at each visit during the Treatment Period. Researchers compared FEV_1 before the 1st dose to FEV_1 at Weeks 8 and 12 of the Treatment Period.

The results below show the average of the change in FEV_1 at Weeks 8 and 12.



What adverse events did the participants have?

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. When new drugs are being studied, researchers keep track of all adverse events participants have, whether or not they are thought to be caused by the trial treatment.

This section is a summary of the adverse events that happened during this trial.

The websites listed at the end of this summary have more information about 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. **Adverse events may or may not be caused by the trial treatment.**

How many participants had adverse events?

The table below shows how many participants had adverse events at any time during the trial.

Summary of adverse events

Participants who:	CSJ117, 0.5 mg 36 participants	CSJ117, 1 mg 37 participants	CSJ117, 2mg 37 participants	CSJ117, 4mg 76 participants	CSJ117, 8 mg 74 participants	Placebo 75 participants
Had at least 1 adverse event	12 of 36 (33%)	14 of 37 (38%)	20 of 37 (54%)	31 of 76 (41%)	23 of 74 (31%)	27 of 75 (36%)
Had at least 1 serious adverse event	1 of 36 (3%)	0 of 37 (0%)	0 of 37 (0%)	1 of 76 (1%)	0 of 74 (0%)	1 of 75 (1%)
Stopped treatment due to an adverse event	1 of 36 (3%)	0 of 37 (0%)	0 of 37 (0%)	0 of 76 (0%)	0 of 74 (0%)	2 of 75 (3%)
Died during the trial	0 of 36 (0%)	0 of 37 (0%)	0 of 37 (0%)	0 of 76 (0%)	0 of 74 (0%)	0 of 75 (0%)

What serious adverse events did the participants have?

A total of 3 participants who received at least 1 dose of trial drug had serious adverse events. The serious adverse events were:

- **CSJ117, 0.5 mg:** 1 out of 36 participants (3%) had COVID-19 and worsening of asthma. Both the events were in the same participant.
- **CSJ117, 4 mg:** 1 out of 76 participants (1%) had worsening asthma.
- **Placebo:** 1 out of 75 participants (1%) had a viral lung infection (Pneumonia viral).

What other adverse events did the participants have?

A total of 106 participants who received at least 1 dose of trial drug had other adverse events. The table below shows the other adverse events that happened in at least 4 participants.

Other adverse events

	CSJ117, 0.5 mg 36 participants	CSJ117, 1 mg 37 participants	CSJ117, 2 mg 37 participants	CSJ117, 4 mg 76 participants	CSJ117, 8 mg 74 participants	Placebo 75 participants
Worsening of asthma **Asthma**	2 of 36 (6%)	4 of 37 (11%)	6 of 37 (16%)	12 of 76 (16%)	7 of 74 (9%)	10 of 75 (13%)
COVID-19	4 of 36 (11%)	2 of 37 (5%)	0 of 37 (0%)	3 of 76 (4%)	1 of 74 (1%)	7 of 75 (9%)
Inflammation of the nose and throat **Nasopharyngitis**	2 of 36 (6%)	2 of 37 (5%)	1 of 37 (3%)	4 of 76 (5%)	2 of 74 (3%)	4 of 75 (5%)
Inflammation of the sinuses **Sinusitis**	1 of 36 (3%)	0 of 37 (0%)	2 of 37 (5%)	1 of 76 (1%)	3 of 74 (4%)	2 of 75 (3%)
Sore throat **Pharyngitis**	0 of 36 (0%)	1 of 37 (3%)	0 of 37 (0%)	2 of 76 (3%)	2 of 74 (3%)	2 of 75 (3%)
Inflammation of the bladder **Cystitis**	0 of 36 (0%)	0 of 37 (0%)	3 of 37 (8%)	3 of 76 (4%)	0 of 74 (0%)	0 of 75 (0%)
Viral infection of the nose, sinuses, and the upper throat **Viral upper respiratory tract infection**	0 of 36 (0%)	0 of 37 (0%)	0 of 37 (0%)	2 of 76 (3%)	2 of 74 (3%)	1 of 75 (1%)
Back pain	0 of 36 (0%)	1 of 37 (3%)	1 of 37 (3%)	2 of 76 (3%)	1 of 74 (1%)	0 of 75 (0%)
Headache	1 of 36 (3%)	0 of 37 (0%)	0 of 37 (0%)	1 of 76 (1%)	1 of 74 (1%)	2 of 75 (3%)
Sudden inflammation of the sinuses **Acute sinusitis**	1 of 36 (3%)	0 of 37 (0%)	2 of 37 (5%)	0 of 76 (0%)	0 of 74 (0%)	1 of 75 (1%)
Inflammation of the airways in the lungs **Bronchitis**	0 of 36 (0%)	1 of 37 (3%)	1 of 37 (3%)	1 of 76 (1%)	1 of 74 (1%)	0 of 75 (0%)
Vaccination complication	0 of 36 (0%)	2 of 37 (5%)	0 of 37 (0%)	1 of 76 (1%)	0 of 74 (0%)	1 of 75 (1%)
Cough	0 of 36 (0%)	0 of 37 (0%)	0 of 37 (0%)	1 of 76 (1%)	3 of 74 (4%)	0 of 75 (0%)

What was learned from this trial?

This trial helped researchers learn about the effects of CSJ117 in people with severe uncontrolled asthma.

The researchers concluded that after 12 weeks of treatment:

- The change in FEV_1 test results between the **CSJ117** study groups and **Placebo** was not big enough to be considered meaningful.
- **CSJ117** was generally safe and no new safety concerns were found.

There are no further studies with CSJ117 planned.

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 **CCSJ117A12201C**

For more information about this trial go to any of the following websites:

- **clinicaltrials.gov** – search using the number **NCT04410523**
- **clinicaltrialsregister.eu/ctr-search/search** – search using the number **2019-004905-29**

Full clinical trial title: A 12-Week, Multicenter, Randomized, Double-Blind, Parallel-Arm, Placebo-Controlled Study to Assess the Efficacy and Safety of CSJ117, When Added to Existing Asthma Therapy in Patients ≥18 Years of Age With Severe Uncontrolled Asthma

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www.novartis.com/clinicaltrials