

The effects and safety of the trial drug CSJ137 in people with anemia who need hemodialysis



Thank you!

Thank you to the participants who took part in the clinical trial for the trial drug **CSJ137**. Every participant helped the researchers learn more about how well CSJ137 works and how safe it is to take.

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: CCSJ137X2201

Drug studied: CSJ137

Sponsor: Novartis

This clinical trial at a glance



What was the purpose of this trial?

[Read more on page 3](#)

The purpose of this trial was:

- To learn if CSJ137 could increase hemoglobin in people who need hemodialysis. Hemoglobin is a protein in the red blood cells that carries oxygen.
- To learn more about the safety of CSJ137.



Who was in this trial?

[Read more on page 4](#)

- 40 men and women participated in this trial.
- The participants were 24 to 84 years old with anemia who needed hemodialysis. Hemodialysis is a treatment to filter waste and water from the blood.



What trial treatment did the participants receive?

[Read more on page 4](#)

- Each participant received CSJ137 one time directly into the blood through an intravenous (IV) infusion after hemodialysis.
- Researchers tested 8 different dose levels of CSJ137 during this trial.



What were the main results of this trial?

[Read more on page 5 to 7](#)

- After receiving CSJ137 one time, some participants showed increased hemoglobin levels in the blood, but most did not. Because of this, researchers concluded that CSJ137 when given one time did not increase hemoglobin levels in the trial participants.
- Most participants had medical problems during this trial. The most common medical problem was nausea. Some of the medical problems were considered to be serious. Some of the serious medical problems seen in this trial are common for people with end-stage kidney disease who need hemodialysis.



You can find **more information** about this trial by going to the websites listed on [page 8 and 9](#).

What was the purpose of this trial?

The purpose of this trial was to see if CSJ137 could treat **anemia** by increasing blood **hemoglobin** in participants with end-stage kidney disease who need hemodialysis.

Hemodialysis is a treatment to filter waste and water from the blood, which is what the kidneys normally do when they are healthy.

These participants had enough iron in their bodies but were unable to use it for making hemoglobin.

Anemia is low levels of hemoglobin in the blood. It is common in people with kidney disease.

Hemoglobin is a protein in red blood cells that carries oxygen. The body needs iron to make hemoglobin.

CSJ137 is a drug designed to release iron from storage in the body to make it available in the blood for making hemoglobin. This is the first trial to test CSJ137 in people. Before a drug can be approved for doctors to prescribe for a condition, researchers do many trials to find out how safe it is and how it works.

The main questions this trial was designed to answer:

- Did the blood hemoglobin levels increase in participants who received CSJ137 one time?
- What medical problems did the participants have during this trial?
Keeping track of the medical problems helped to learn about the safety of CSJ137.

How long was this trial?

This trial took place from September 2015 to May 2020. The trial was planned to last up to 6 months for each participant.

This trial ended early. During the trial, researchers found that the hemoglobin levels in participants' blood varied because of hemodialysis. So, it was not possible to learn if CSJ137 could increase the blood hemoglobin levels. The decision to end the trial was not based on the safety results.

Who was in this trial?

40 participants received treatment – 22 men and 18 women. 1 participant did not complete this trial. The participants were 24 to 84 years old. Their average age was 59 years.

Each participant in this trial had end-stage kidney disease and low levels of hemoglobin in their blood. They also:

- Needed hemodialysis
- Received a protein called erythropoietin, which helped the body to make hemoglobin



This trial took place in Israel and the United States.



Visit novctrd.com for more information about:

- Who could and could not be in this trial
- The participants in this trial, such as their age, gender, and race
- Reasons why participants did not complete the trial

Use trial number **CCSJ137X2201** to find the scientific summary.

What trial treatment did the participants receive?



This trial was planned to have 2 parts, but the trial ended during Part 1.

During **Part 1**, the researchers and participants knew which treatment was given to each participant.

CCSJ137: The participants received CSJ137 one time directly into the blood through an IV infusion that lasted for about 30 minutes. CSJ137 was given after hemodialysis.

Researchers tested 8 different dose levels of CSJ137 to find out if different amounts of CSJ137 could increase hemoglobin levels. Each participant received only 1 dose level of CSJ137.

What were the main results of this trial?



This is a summary of the overall results of this trial. Individual results from each participant may be different and are not included in this summary.

Researchers need many trials to learn if a drug or other treatment is safe and works well. Other trials may provide new information or different results.

Always talk to a doctor before making any changes to your health care.

Did the blood hemoglobin levels increase in participants who received CSJ137 one time?

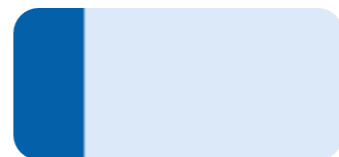


After receiving CSJ137 one time, some participants showed increased hemoglobin levels in the blood, but most did not. Because of this, researchers concluded that CSJ137 when given one time did not increase hemoglobin levels in the trial participants.

To find this out, researchers collected blood samples from the participants. Researchers checked if participants had an increase in blood hemoglobin levels on Day 29 compared to the start of the trial. For this question, the results were available for 38 participants only.

**Participants who had an increase
in blood hemoglobin levels**


21% (8 of 38)



You can find more information about these results by going to the websites listed on [page 8](#) and [9](#).


What medical problems did the participants have during the trial?

Medical problems that happen during trials are called “adverse events”. An **adverse event** is any unwanted sign or symptom that the participants have during a trial. An adverse event is 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.

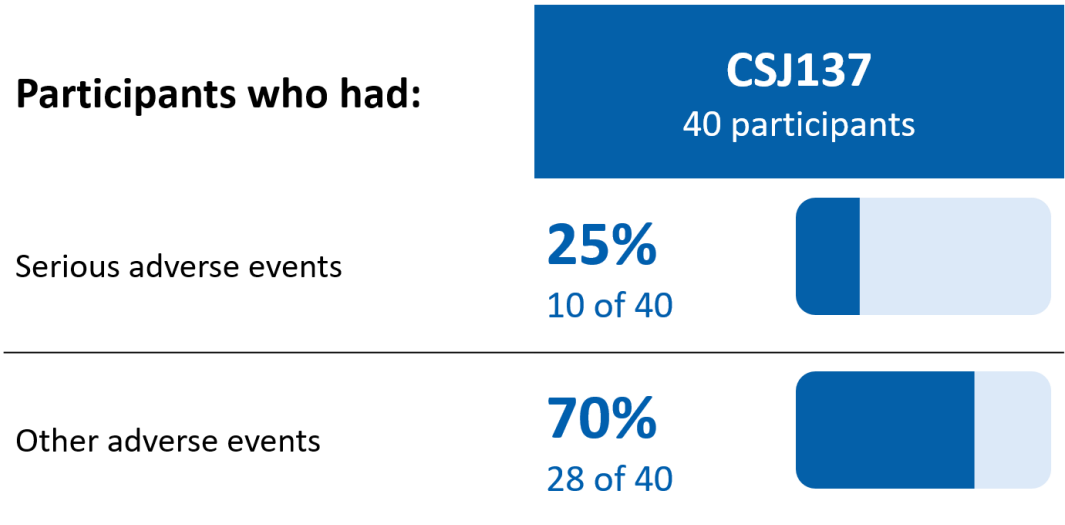
Many trials are needed to know if a drug or treatment causes an adverse event. Trial doctors keep track of all adverse events that happen in trials, even if they think the adverse events might not be related to the trial treatments.



Most participants had adverse events during this trial. At each dose level, a similar number of participants had adverse events. The most common adverse event was nausea.

Some of the adverse events were considered serious. Some of the serious adverse events seen in this trial are common for people with end-stage kidney disease who need hemodialysis.

Participants who had adverse events



None of the participants left this trial due to adverse events.

What serious adverse events did the participants have?

18 serious adverse events occurred in total of 10 participants.

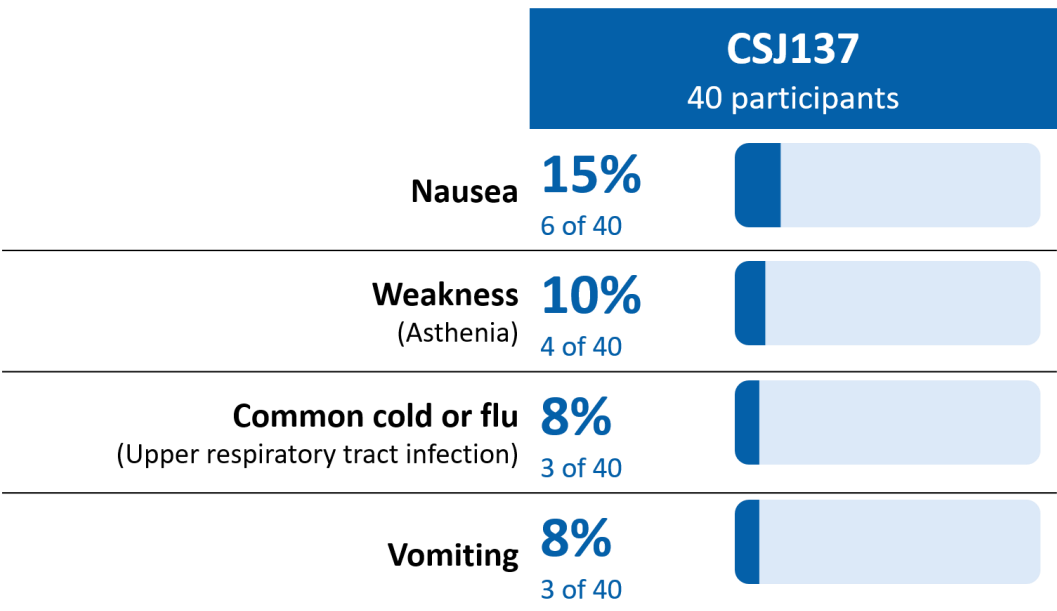
The most common serious adverse event was:

- Damage in the heart's blood vessels (coronary artery disease) that occurred in 2 participants.

The rest of the events were reported by 1 participant each. No participant died during this trial.

What other adverse events did the participants have?

Most participants in this trial had adverse events that were not serious. The table below shows the adverse events that happened to **3 or more participants**. Other adverse events were reported by fewer participants.



For more information about the adverse events the participants in this trial had, visit novctrd.com. Use trial number **CCSJ137X2201** to find the scientific summary.

What was learned from this trial?

This was the first trial in people to learn about the safety and effects of CSJ137.

The researchers learned about the safety of CSJ137 which may help with future trials. The researchers concluded that the serious adverse events that occurred during this trial are common for people with end-stage kidney disease who need hemodialysis.

The researchers learned that it was not possible to fully understand the effect of CSJ137 in this population because:

- Hemodialysis caused hemoglobin levels to vary
- Each participant received CSJ137 only one time



The results presented here are for one trial. One trial cannot give a complete picture of the benefits and risks of a trial drug. The results of many trials are needed to find out which treatments can be used for people with end-stage kidney disease who need hemodialysis. This summary shows only the main results from this trial. Other trials may provide new information or different results.

Where can I learn more about this and future clinical trials?



You can find detailed results and more information about this clinical trial on the Novartis Clinical Trial Results website:

1. Visit novctrd.com
2. Click on “Clinical Trial Results” at the top right of the page
3. Read and scroll down, then click “I accept” to agree to use the information and the website
4. Select “study number” from the drop-down menu
5. Type “**CCSJ137X2201**” in the search box and click search

If you would like to view the website in a language other than English, you can click the “Google Translate” button on the top right corner of the page.



If you were in this trial and have **questions** about the results, please speak with the doctor or staff at your trial site.

This trial was registered on the following websites:

- ClinicalTrials.gov – <https://clinicaltrials.gov/>
To find this trial, type CCSJ137X2201 in the **Other terms** search box
- European Union Clinical Trials Register – <https://www.clinicaltrialsregister.eu/ctr-search>
To find this trial, type **CCSJ137X2201** in the search box

Full trial title:

A first-in-human, two-part (open label, and randomized/double blind/placebo controlled), single- and repeat-dose study of CSJ137 in erythropoietin-treated chronic hemodialysis patients with functional iron-deficiency anemia

If more trials are planned, they will appear on the public websites listed above. When there, search for **CSJ137**.

Thank you!

Novartis would like to thank all of the people who participated in this clinical trial. The participants made this clinical trial possible and helped researchers answer important health questions and learn about a possible medical treatment. Many volunteers and many clinical trials are needed to advance medical science.



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

1-888-669-6682 (USA)
+41-61-324 1111 (EU)
www.novartisclinicaltrials.com