Clinical Trial Results Summary

Research Sponsor: Novartis

Drug Studied: SOM230 (pasireotide)

Trial Number: CSOM230Y2201

Plain Language Title: A trial to learn about the effects and safety of SOM230 in participants with cluster headaches

Thank you!

Thank you to the participants who took part in the clinical trial for the trial drug SOM230, also known as pasireotide. All of the participants helped the researchers learn more about how SOM230 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. An independent organization prepared this summary of the trial results.

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

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

You can find more information about this trial on the website listed on the last page of this summary.

Overview of this trial

What was the purpose of this trial?

This clinical trial was designed to learn if the trial drug SOM230 reduced the severity of pain of participants with cluster headaches. The clinical trial team also wanted to study the safety of SOM230 in these participants.

What treatments did the participants take?

The participants took a single dose of both SOM230 and a placebo. A placebo looks like the trial drug but does not have any trial drug in it. SOM230 and the placebo were given as injections under the skin.

Who took part in the trial?

28 men and women with cluster headaches participated in this clinical trial.

What did the researchers want to learn?

The main questions the researchers wanted to answer in this trial were:

- Did SOM230 reduce the severity of a cluster headache in the participants?
- What medical problems did the participants have during the trial?

Keeping track of the participants' medical problems helped the researchers learn more about the safety of SOM230.

What were the main results of the trial?

Overall, the researchers learned that:

- SOM230 did not help reduce the severity of a cluster headache more than the placebo.
- Most of the participants had medical problems during this trial, and none of the medical problems were serious. The most common medical problems were nausea and vomiting.

Details of medical problems are listed beginning on page 8.

Why was the research needed?

Researchers are looking for a better way to treat patients with cluster headaches. Before a drug can be approved for patients to take, researchers do clinical trials to find out how safe it is and how it works. In this trial, the researchers studied how the trial drug called SOM230 affected the severity of the participants' cluster headaches. The researchers also studied the safety of SOM230.

People with cluster headaches can have many severe and painful headaches that often happen only on 1 side of their head. These headaches can happen every day, many times a day, and they can also last for days, weeks, or months. These cycles of time are called "clusters". Cluster headaches may happen around the same time each year, often in the spring and the fall.

The cause of cluster headaches is unknown. Current treatments include the use of oxygen masks and a drug called sumatriptan. But, these treatments do not help some people with cluster headaches, and their effects do not last for a long time.

The trial drug, SOM230, works by acting like a hormone in the body known as somatostatin. Researchers think that somatostatin may help the pain in cluster headaches by decreasing the activity of cells in the nervous system that play a role in pain. The nervous system is made up of the brain, spinal cord, and nerves throughout the body.

In another trial, a drug that works like SOM230 helped reduce the severity of cluster headaches in those trial participants, but its effects lasted only a very short time. The drug used in that trial had to be given through a needle into a vein, called an IV infusion.

In this trial, the researchers wanted to learn if SOM230 could treat cluster headaches when given through a needle put under the skin, called a subcutaneous injection, also called an SC injection. SC injections can be taken at home, compared to IV infusions that have to be given in a clinic.

What was the purpose of the trial?

The main questions the researchers wanted to answer in this trial were:

- Did SOM230 reduce the severity of a cluster headache in the participants?
- What medical problems did the participants have during the trial?

What treatments did the participants take?

The participants in this trial took both SOM230 and a placebo through an SC injection. A placebo looks like the trial drug but does not have any trial drug in it. During the trial, when a participant had their first cluster headache, they took the placebo. When they had their second cluster headache, they took SOM230. The dose of SOM230 was measured in milligrams, also called mg.

The chart below shows the treatments the participants took.

	First headache	Second headache
	• 28 participants	• 26 participants
	• The placebo	• 1.5 mg of SOM230
	• Through an injection under the skin	
	• A single dose	

Who took part in the trial?

To answer the questions in this trial, the researchers asked for the help of 28 men and women with cluster headaches. The participants' ages ranged from 24 to 60 years old. They were 44 years old on average.

The trial took place in 3 countries: Germany, the United Kingdom, and the United States.

What type of trial was this?

This trial studied the trial drug's safety and how well it worked in a small number of participants.

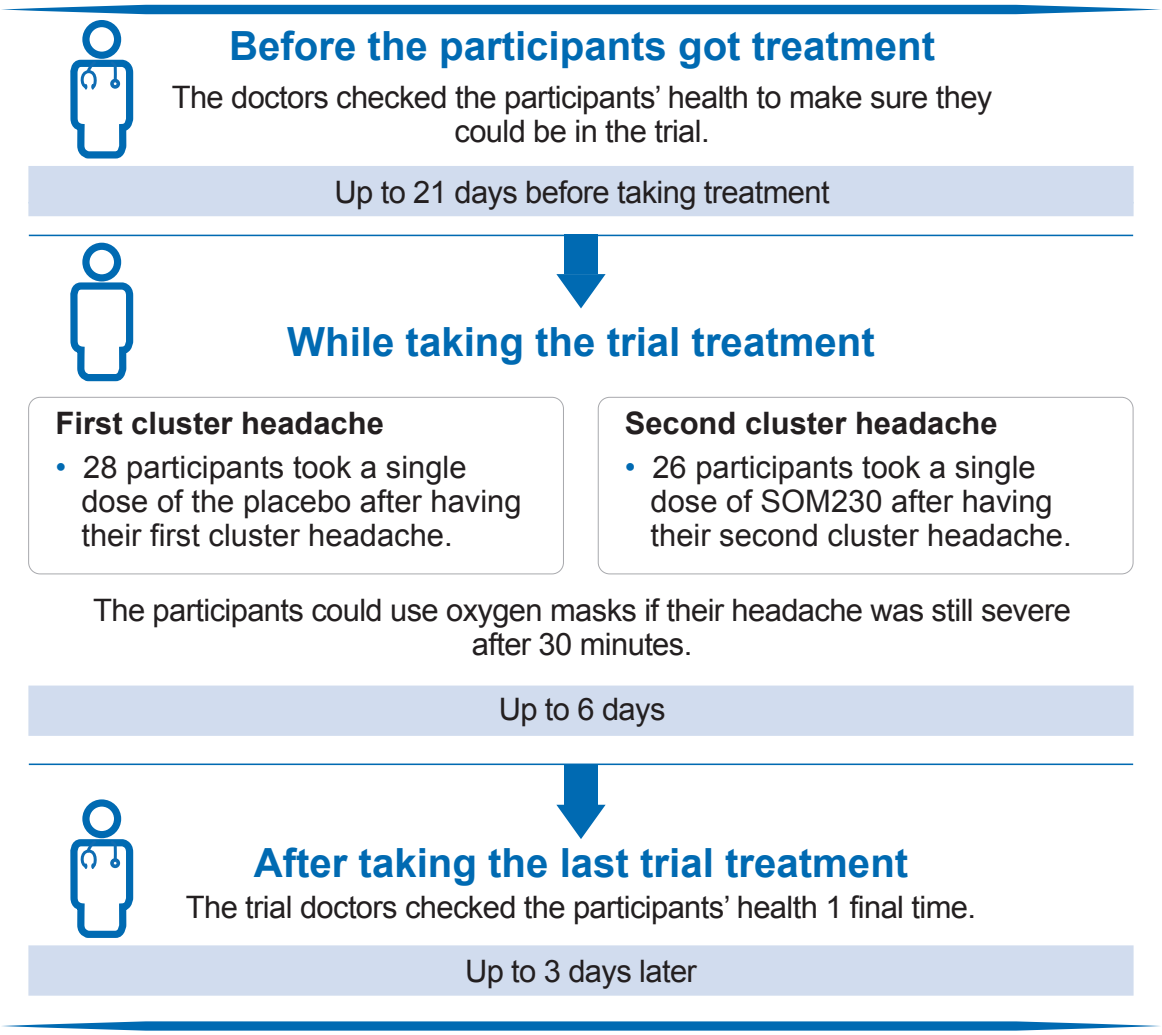
During this trial, the trial staff and sponsor staff knew what each participant was taking, but the participants did not.

What happened during the trial?

Each participant was in the trial for up to 30 days. The trial started in October 2016 and ended in September 2018.

Partway through the trial, the researchers reviewed the data. The trial ended early because the results showed that SOM230 did not help the participants as much as expected.

The chart below shows what happened during the trial.



What did researchers learn from the results of the trial?

This is a summary of the overall results from this trial. The individual results of each participant might be different and are not in this summary.

Other trials may provide new information or different results. Always talk to a doctor before making any changes to your health care.

Did SOM230 reduce the severity of a cluster headache in the participants?

SOM230 did not help reduce the severity of a cluster headache more than the placebo.

To answer this question, the trial doctors asked the participants to take the treatment at the beginning of a cluster headache. The participants took treatment only when their pain was moderate or worse. The participants reported the following in their electronic diaries:

- the severity of their pain before and after taking the trial treatment
- if their headache became mild or went away 30 minutes after taking the treatment
- if they needed to take 100% oxygen 30 minutes after taking the trial treatment because of pain
- if they took any other medication during the cluster headache

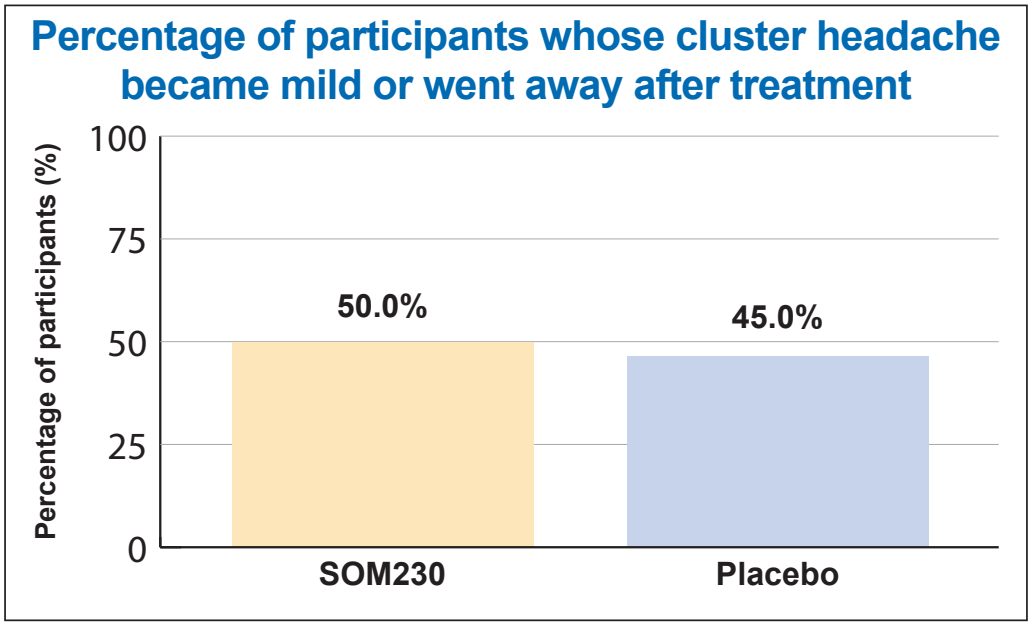
Based on the diary entries, the researchers counted the number of participants whose headache became less severe. They counted how many participants took their treatment without having to use any other medicines within 30 minutes to help their pain. This included 20 out of the 28 participants. The remaining 8 participants needed other medicines to treat their pain, so the researchers could not use their results. This was because, the researchers could not be sure if the severity of the participants' pain went down because of the trial treatment or the other medicine.

Overall, the researchers found there were participants who had a less severe cluster headache after taking both SOM230 and the placebo. But, the difference between the 2 treatments was too small for the researchers to conclude that SOM230 helped reduce the severity of the cluster headache more than the placebo.

The percentage of participants whose headache became mild or went away 30 minutes after treatment was:

- 50.0% of the participants when they took SOM230. This was 10 out of the 20 participants.
- 45.0% of the participants when they took the placebo. This was 9 out of the 20 participants.

The chart below shows these results.



What medical problems happened during the trial?

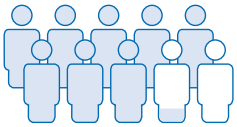
Medical problems that happen in clinical trials are called “adverse events”. An **adverse event** is any unwanted sign or symptom that participants have during a trial. An adverse event is considered “serious” when it is life-threatening, causes lasting problems, or the participant needs hospital care.

Adverse events may or may not be caused by the treatments in the trial. A lot of research is needed to know whether a treatment causes an adverse event. Doctors keep track of all the adverse events that happen in trials, even if they do not think the adverse events might be related to the treatments.

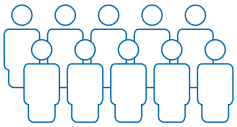
This section is a summary of the adverse events that happened during this trial.

Most of the participants had adverse events during this trial, and none of the adverse events were serious. The most common adverse events were nausea and vomiting.

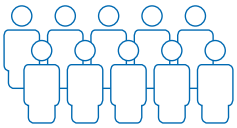
Summary of adverse events



- 82.1% of participants had adverse events. This was 23 out of the 28 participants.



- None of the participants had serious adverse events.



- None of the participants stopped treatment because of adverse events.

What were the most common serious adverse events?

None of the participants had serious adverse events or died during this trial.

What were the most common adverse events?

Many of the adverse events that happened in this trial were considered typical for the way SOM230 works in the body. The most common adverse events during this trial were nausea and vomiting.

The adverse events below happened in 3 or more participants during the trial. There were other adverse events, but these happened in fewer participants.

Most common adverse events in this trial

	1.5 mg of SOM230 (Out of 26 participants)	Placebo (Out of 28 participants)	Total (Out of 28 participants)
Nausea	46.2% (12)	0.0% (0)	42.9% (12)
Vomiting	26.9% (7)	3.6% (1)	28.6% (8)
Diarrhea	26.9% (7)	0.0% (0)	25.0% (7)
Tiredness	23.1% (6)	3.6% (1)	25.0% (7)
Pain where the needle was put in	19.2% (5)	3.6% (1)	21.4% (6)
Redness where the needle was put in	15.4% (4)	3.6% (1)	17.9% (5)
Stomach pain	11.5% (3)	0.0% (0)	10.7% (3)

For more information about the adverse events in this trial, please see the scientific summary that can be found on the website noted at the end of the summary.

What was learned from this trial?

The information described above helped researchers learn if SOM230 helped reduce the severity of headaches and about its safety in participants with cluster headaches.

More research is needed to find out which treatments can be used for patients with cluster headaches. This summary shows only the main results from this one trial. Other trials may provide new information or different results.

Where can I learn more about this trial?

More information about the results of this trial can be found in the scientific results summary available on the Novartis Clinical Trial Results website.

- Go to www.novctrd.com.
- Once on the site, click **"Clinical trial results and trial summary for patients"** at the top right of the page.
- After accepting the terms, go to the bottom left of the page and click **"Search by study number"**.
- Type **"CSOM230Y2201"** into the keyword 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 of the page.

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

You can find more information about this trial on the websites listed below.

- www.clinicaltrials.gov Once you are on the website, type **"CSOM230Y2201"** into the **"Other terms"** search box and click **"Search"**.

If you would like to search by trial title, please use the full trial title listed below.

- www.clinicaltrialsregister.eu Once you are on the website, click **"Home and Search"**, then type **"CSOM230Y2201"** in the search box and click **"Search"**.

Novartis does not have further clinical trials in cluster headaches planned at this time. If you are interested in finding other trials in cluster headaches or trials with SOM230 they will be listed on the above public websites.

Full trial title: A multicenter, placebo-controlled, single dose study in acute episodic and chronic cluster headache to evaluate the safety and efficacy of SOM230 subcutaneous (s.c.)

Protocol number: CSOM230Y2201

Thank you!

Clinical trial participants belong to a large community of participants around the world. They help researchers answer important health questions and test new medical treatments.

NOVARTIS

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