

**Research Sponsor:** Novartis

**Drug Studied:** CFZ533

**Protocol #:** CCFZ533X2205

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## ***Thank you!***

Thank you for taking part in the clinical trial for the drug CFZ533. You and all of the patients helped researchers learn more about how CFZ533 works in people with Graves' disease, also called GD.

Novartis sponsored this trial and believes it is important to share the results of the trial with you and the public. An independent non-profit organization called CISCRP prepared this summary of the trial results for you. We hope it helps you understand your important role in medical research.

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

## **What has happened since the trial ended?**

You were in this trial for up to 9 months. But, the entire trial took 1 year to finish. This is because patients started and stopped at different times. The trial started in April 2016 and ended in April 2017.

The trial included 15 patients from 3 trial sites in Germany and the United States. After the trial ended, the sponsor reviewed the data and created a report of the results. This is a summary of that report.

## Why was the research needed?

Researchers are looking for a better way to treat people who have GD. Before a drug can be approved for patients to take, researchers do clinical trials to find out how well it works and how safe it is. This information is important to know before other trials can be done to find out if CFZ533 improves the health of people with GD.

GD is an immune system disease that affects the thyroid gland. The thyroid gland makes proteins called hormones. Thyroid hormones control things like how fast the heart beats and how fast calories are burned. In people with GD, the immune system attacks the thyroid and causes it to make higher than normal levels of thyroid hormones. High levels of thyroid hormones in the blood can lead to serious medical problems.

CFZ533 was designed to lower the activity of the immune system. The researchers in this trial wanted to learn if CFZ533 could lower the activity of the immune system in patients with GD enough to help their thyroid make normal levels of thyroid hormones.

In this trial, the researchers wanted to find out if CFZ533 works in a small number of patients with GD. Other treatments for GD are available, but they can have harmful long term effects, or the GD can come back. So, researchers are looking for a better way to treat GD.

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

- Did the patients' thyroid hormones reach normal levels after taking CFZ533?
- What medical problems did patients have?

## What kind of trial was this?

To answer the questions in this trial, the researchers asked for the help of men and women with GD. The patients in this trial were 21 to 65 years old.

This was an “open-label” trial. This means each patient knew what they were getting. The trial staff and sponsor staff also knew what each patient was getting.

## What happened during the trial?

**Before treatment started**, the trial doctors did tests and checked the health of the patients to make sure they could take part in the trial. The patients gave blood and urine samples. The trial doctors also checked the heart health of the patients using an electrocardiogram, also called an ECG.

The trial doctors asked patients to stop taking certain medicines. This helped the researchers make sure any effects they saw during the treatment part of the trial were due to the trial drug and not due to other medicines.

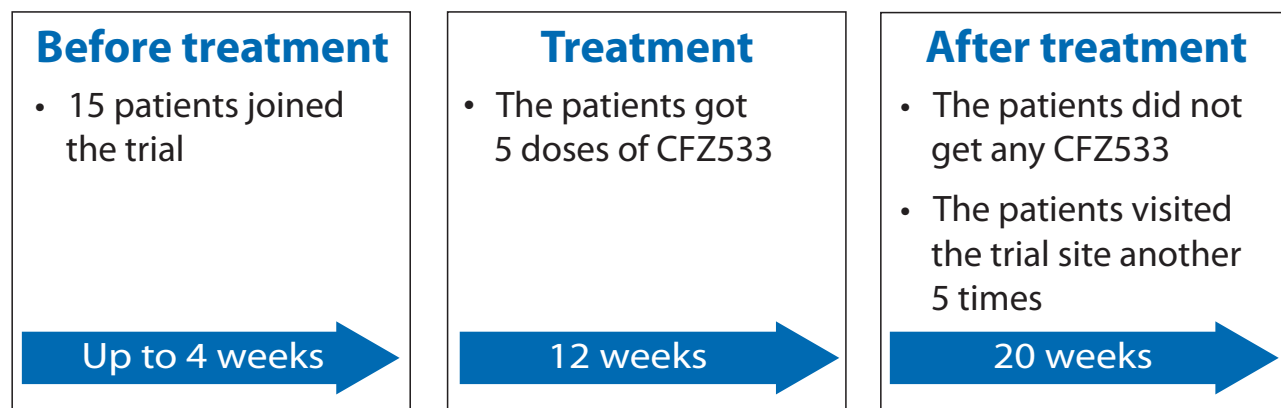
**During treatment**, the patients got a total of 5 doses of CFZ533 at the trial site over about 12 weeks. The dose each patient got was based on his or her body weight. Doses were measured in milligrams per kilogram of body weight, also called mg/kg. All the patients got 10 mg of CFZ533 for every kg of their body weight. They got the trial drug through a needle into a vein.

Throughout the trial, the trial staff:

- Checked the overall health of the patients
- Took blood and urine samples
- Asked questions about how the patients felt and about any other medicines they were taking

**After treatment**, the patients visited the trial site another 5 times over 20 weeks. At each visit, the trial doctors checked the overall health and heart health of the patients. The trial doctors also checked how the patients were feeling and what medicines they were taking. The patients gave more blood and urine samples.

The chart below shows how the trial was done.



## What were the results of the trial?

This is a summary of the overall results of this trial, not your individual results. The results presented here are for a single trial. Researchers look at the results of many trials to decide which drugs work best and are safest for patients. Other trials may provide new information or different results. You should not make medical decisions based on the results of a single trial without first talking to your doctor. Always talk to a doctor before making any changes to your medications or treatment plans.

During the trial, 2 patients got rescue medicine. This is medicine that patients could get if they needed urgent help for their GD symptoms. In these patients, the researchers could not be sure if the effects seen during the trial were caused by the trial drug or the rescue medicine. So, the researchers did not include these 2 patients in some of the results.

### Did the patients' thyroid hormones reach normal levels after taking CFZ533?

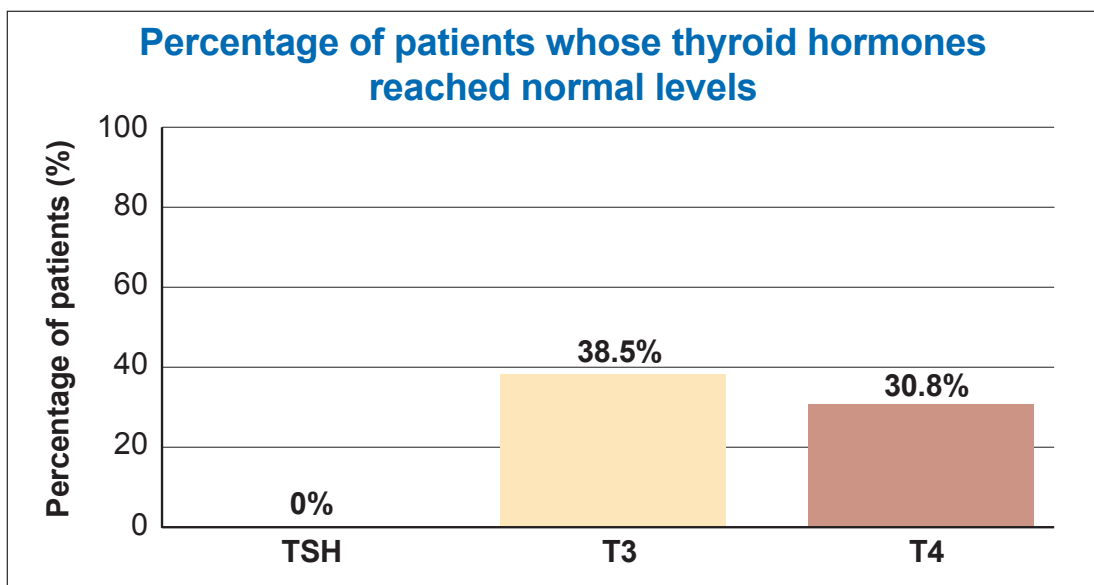
The researchers wanted to know if CFZ533 affected how the patients' thyroids worked. To find this out, the researchers counted the number of patients whose thyroid hormones reached a normal range after 12 weeks of treatment.

To do this, the trial doctors measured 3 thyroid hormones that are found in the blood. If these hormones reached normal levels, it meant that the patients' thyroids were working normally. The researchers measured the thyroid hormones called TSH, T3, and T4. The researchers measured these thyroid hormones throughout the trial.

After 12 weeks of treatment, the researchers found that:

- None of the patients had their TSH reach normal levels.
- 38.5% of the patients had their T3 reach normal levels. This was 5 out of 13 patients.
- 30.8% of the patients had their T4 reach normal levels. This was 4 out of 13 patients.

The chart below shows the percentage of patients who had their thyroid hormones reach normal levels after 12 weeks.



After treatment ended, the researchers continued to check the thyroid hormone levels of all 15 patients. The researchers learned that:

- In 7 out of 15 patients, at least 1 of the thyroid hormones reached normal levels after 24 weeks. This was 12 weeks after the last dose of CFZ533.
- Two out of 15 patients had normal levels of TSH by week 24. This was 12 weeks after the last dose of CFZ533.

TSH can take at least 2-3 months longer than the other thyroid hormones to reach normal levels after treatment.

## What medical problems did patients have?

Medical problems that happen in clinical trials are called “adverse events”. An adverse event is any unwanted sign or symptom that patients have during a trial. An adverse event is considered “serious” when it is life-threatening, causes lasting problems, or the patient needs hospital care. These problems may or may not be caused by the trial drug.

A lot of research is needed to know whether a drug causes a medical problem. During a trial, all medical problems are reported and written down, whether or not they are caused by the trial drug. So, when new drugs are being studied, researchers keep track of all medical problems that patients have.

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

### How many patients had adverse events?

The table below shows how many patients had adverse events during this trial.

Adverse events in this trial		
How many patients in this trial had adverse events? (Out of 15 patients)	How many patients in this trial had serious adverse events? (Out of 15 patients)	How many patients left this trial because of adverse events? (Out of 15 patients)
80.0% (12)	6.7% (1)	0.0% (0)

### What were the most common serious adverse events?

One patient experienced the serious adverse event of rapid heart rate. This was 6.7% of patients. The doctors thought this serious adverse event was caused by the patient’s GD. Rapid heart rate is common in patients who have GD.

None of the patients died during this trial.

## What were the most common adverse events?

Inflammation of the bladder or urinary tract was the most common adverse event in this trial. The table below shows the most common adverse events that happened in 10.0% or more of patients. There were other adverse events, but these happened in fewer patients.

Most common adverse events in this trial	
Adverse event	CFZ533 (Out of 15 patients)
Inflammation of the bladder or urinary tract	20.0% (3)
Headache	13.3% (2)
Insomnia	13.3% (2)
Nausea	13.3% (2)
Tiredness	13.3% (2)
Upper respiratory tract infection	13.3% (2)
Upper respiratory tract infection caused by a virus	13.3% (2)

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

## How has this trial helped patients and researchers?

This was the first time that researchers studied CFZ533 in patients with GD. The results of this trial helped researchers better understand if CFZ533 can help the thyroid work normally. This research helped doctors understand more about CFZ533 as a possible treatment for GD.

The results from several trials are needed to decide which treatments work best and are safest. This summary shows only the main results from this 1 trial. This trial was done in a small number of patients over a short time period. 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 ([www.novctrd.com](http://www.novctrd.com)). Once on the site, click **“Read More”** under **“Clinical trial results”** at the bottom of the page. After agreeing to enter the Novartis website, type **“CCFZ533X2205”** into the keyword search box and click **“Search”**. If you 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](http://www.clinicaltrials.gov). Once you are on the website, type **“NCT02713256”** into the search box and click **“Search”**.
- [www.clinicaltrialsregister.eu](http://www.clinicaltrialsregister.eu). Once you are on the website, click **“Home and Search”**, then type **“2015-005564-41”** in the search box and click **“Search”**.

If more clinical trials are planned, they will be listed on the above public websites or [www.novartisclinicaltrials.com](http://www.novartisclinicaltrials.com). Search for **“CFZ533”**.

**Full Trial Title:** An open label study to evaluate the safety and efficacy of 12 week treatment with CFZ533 in patients with Graves’ disease

## Thank you

As a clinical trial participant, you belong to a large community of participants around the world. You helped researchers answer important health questions and test new medical treatments.



The Center for Information & Study on Clinical Research Participation (CISCRP) is a non-profit organization focused on educating and informing the public about clinical research participation.

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