Clinical Trial Summary

A clinical trial to test the drug LFG316 in patients with non-infectious uveitis

Thank you!

Novartis, the sponsor of this clinical trial, thanks the patients who helped make this clinical trial possible.

Overview of results

Protocol: CLFG316A2204

In this clinical trial, researchers studied how symptoms of patients with non-infectious uveitis responded to a drug named LFG316. Non-infectious uveitis can cause pain and redness in the eye, blurred or loss of vision, eyes that are sensitive to light, and spots in vision (floaters). Non-infectious means the uveitis is not caused by an infection from a virus, bacteria, or fungus.

What should I know about clinical trials?

A clinical trial is a type of research designed to learn more about how our bodies respond to drugs or other treatments. Do not use the results of only one clinical trial to make decisions about your health care.

The researchers wanted to study:

- If the trial drug LFG316 had an effect on the symptoms of uveitis during and after 85 days of treatment
- How much LFG316 got into patients' bloodstream

Who was in this clinical trial?

25 male and female adults with uveitis began this clinical trial.

What type of clinical trial was this?

This was a Phase 2 clinical trial, which tests a trial drug's safety and how well it works in a small number of patients.

How was safety tested?

Researchers looked for any medical problems, called adverse events, that happened during this clinical trial. Patients reported adverse events, including 3 serious adverse events. The doctors at each trial site did not believe LFG316 caused these serious adverse events.

What did researchers learn in this clinical trial?

LFG316 did not help the symptoms of non-infectious uveitis.

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Researchers need many clinical trials to learn if a drug or other treatment is safe and works well. This is a summary of the overall results of this clinical trial, not the results for each patient in the clinical trial. Do not use only the results of this clinical trial to make health decisions. Always talk to a doctor before making any changes to your treatments.

What do I need to know about this clinical trial?

The researchers that led this clinical trial decided that LFG316 did not cause any changes in the symptoms of uveitis for any patients.

Why was this clinical trial needed?

Researchers are looking for a better way to treat non-infectious uveitis. Uveitis is an eye disease that can cause:

- Pain and redness in the eye (often called inflammation)
- Blurred or loss of vision that can be permanent
- Eyes that are sensitive to light
- Spots, specks, or lines floating in vision (floaters)

Non-infectious means the uveitis is not caused by an infection from a virus, bacteria, or fungus.

Researchers think the body's immune system may be a cause of non-infectious uveitis. The body's immune system is made of cells and proteins that defend the body from illness and infections. Sometimes, the immune system can attack the body's healthy cells instead of protecting them. This can cause inflammation. Researchers think an immune system protein called C5 may play a role in causing the inflammation and other symptoms of uveitis.

Researchers designed this clinical trial to learn if the trial drug LFG316 could lessen the symptoms of uveitis by stopping C5 from signaling the immune system. Researchers compared the trial drug to standard treatments that people usually get for uveitis, such as steroids. The doctor at each clinical trial site decided the standard treatment for each patient.

In this clinical trial, the researchers wanted to learn:

- If the trial drug LFG316 had an effect on the symptoms of uveitis during and after 85 days of treatment
- How much LFG316 got into patients' bloodstream
- About any medical problems, called adverse events, that patients reported

Who was in this clinical trial?

25 adult patients – 14 male and 11 female – who were 44-years-old on average began this clinical trial. Researchers planned the main time of treatment to last 85 days for each patient.

All patients in this clinical trial had at least one eye with non-infectious uveitis and were:

- Between 19 and 76 years of age
- In good overall health
- Not being successfully treated for their non-infectious uveitis
- Taking stronger than usual treatment to try and lower their immune system's activity

Patients who received LFG316 in this trial also agreed to lessen or stop the treatment to lower their immune system's activity.

Researchers conducted this trial at 10 health care centers in the United Kingdom and the United States.

Not all patients completed the trial as planned:

- 2 patients stopped being part of this clinical trial
- 3 patients stopped getting LFG316, but still completed visits to the health care center for the clinical trial
- 23 patients completed the 85-day treatment

What type of clinical trial was this?

This was a Phase 2 clinical trial, which tests the safety of a trial drug and how well it works in a small number of patients. This was the first time that researchers tested how well LFG316 works in patients with uveitis. Researchers also looked for any medical problems that patients had during the study.

Researchers used a computer program to randomly choose the treatment for each patient. The research staff who tested patients' eyes did not know which treatment was given to a patient. Researchers do this so that comparing the results of each treatment is done as fairly as possible. The patients, some researchers, and the sponsor did know if a patient got a standard treatment or LFG316.

Each patient started at different dates during the trial. This trial began in December 2012 and ended in August 2017.

What happened during this clinical trial?

Before the trial treatment started

Researchers tested patients to make sure they could be in this trial. Researchers also asked patients to stop or take less of some medicines based on which trial treatment they would be given.

During treatment

Researchers gave patients either:

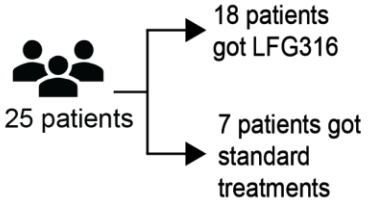
- **LFG316:** A total of 3 shots (injections) in one eye. Researchers gave each patient one shot every 4 weeks.
- **Standard treatment**: The doctor at each trial site decided which standard treatment, such as steroids, to give each patient.

During the 85-day long treatment, researchers asked all patients to have both their eyes checked up to 8 different times at the trial health care center.

Extended treatment

At day 85, researchers asked 5 patients who may have responded to LFG316 if they would continue treatment. 4 of the 5 patients continued getting LFG316 for about 6 more months. This summary reports the main results from participants in the 85-day-long part of this clinical trial.

How researchers designed this clinical trial:

Before treatment	During treatment	End of main treatment	Extended treatment
• Researchers tested patients to make sure they could be in this trial • Researchers asked patients to stop or take less of some medicines		Most patients had their last visit and test of their eyes on day 85	Researchers asked 5 patients to continue for about 6 more months
Up to 14 days	**85 days**	**After 85 days**	**196 more days**

What did researchers learn in this clinical trial?

This is a summary of the overall results of this clinical trial, not the individual results of each patient. This is a summary of only one clinical trial. Do not use only the results of this clinical trial to make decisions about your health care.

Always talk to a doctor before making any changes to your treatments.

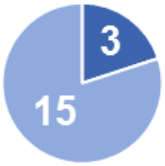
Researchers learned LFG316 did not help the symptoms of non-infectious uveitis.

To find this out, researchers checked patients' eyes for:

- How many letters a patient could read on an eye chart
- How cloudy the liquid in their eye was
- How many cells appeared due to inflammation
- The number of spots or scars on their retina, which is the back part of the eye that is sensitive to light
- If their uveitis got worse

During the trial, researchers found that 2 patients needed more medicine for their uveitis. Also during the trial, some patients did not go to all of the planned visits to test their eyes. For all patients who did not exactly follow the research plan, researchers cannot tell what caused any changes that happened during the trial. After 85 days, some patients had at least 1 change in the symptoms of uveitis, and some had no symptoms of uveitis.

Patients who had at least 1 change in the symptoms of uveitis at the end of the clinical trial



3 of **18** (17%) of the patients on **LFG316**

3 of **7** (43%) of the patients on **standard treatments**

These patients had at least one of these changes by the end of the clinical trial:
Could read at least 10 more letters from an eye chart
Liquid in their eye became less cloudy
Fewer cells appeared due to inflammation
Spots or scars on their retina went away

Patients who had almost no symptoms of uveitis at the end of the clinical trial

2 of **18** (11%) of the patients on **LFG316**

0 of **7** (0%) of the patients on **standard treatments**

These patients had all of the following changes by the end of the clinical trial:
Liquid in their eye is clear or only a little cloudy
Very few or no cells appeared due to inflammation
No spots or scars on their retina
Didn't take other immune system treatments
Uveitis did not get worse

Even though 2 of the 18 patients on LFG316 had almost no symptoms of uveitis at the end of the clinical trial, this was too small of a number of patients for researchers to say that the trial drug worked compared to standard treatments. Also, no patient began the clinical trial with all of the symptoms of uveitis. Therefore, the researchers decided that LFG316 did not cause any of the changes in the symptoms of uveitis.

What other key results did researchers learn?

In this clinical trial, researchers also learned:

- **If LFG316 had effects on patients' eyes at any of the visits during the trial**

  Researchers looked at each patient's eyes up to 8 times during the trial to see if there were any effects. The researchers did not see a difference between patients that got LFG316 and patients that got standard treatments.

- **How much LFG316 got into patients' bloodstream**

  Researchers wanted to know if the trial drug would leave the eye and enter into the patients' bloodstream. They found small amounts of LFG316 in the blood, but found that it didn't build up or cause any effects.

What medical problems did patients report?

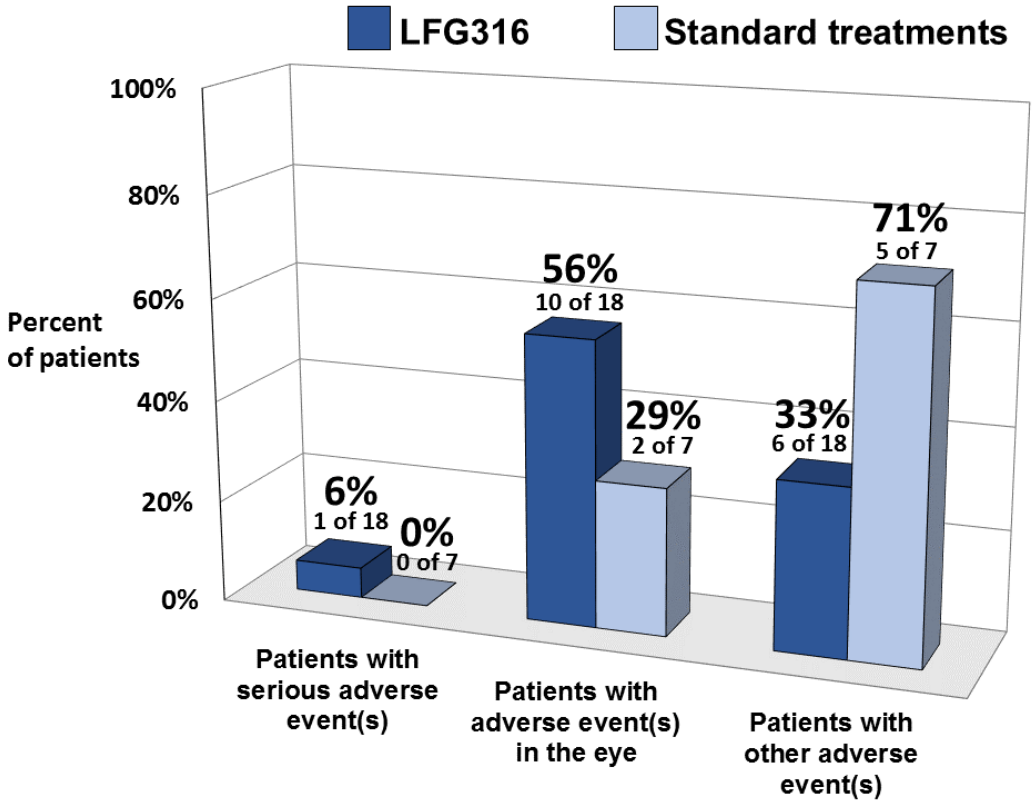
Medical problems that occur during a clinical trial are called adverse events. An adverse event is any unwanted sign or symptom that patients report during a trial. These problems may or may not be caused by the clinical trial or the trial drug. Researchers need many trials to know if a drug causes a medical problem. Researchers ask patients to report all adverse events during a trial.

During the clinical trial, patients reported adverse events, including 3 serious adverse events. Researchers kept track of the adverse events and whether or not they thought LFG316 was the cause. Researchers also looked at which adverse events were related to the eye and which were not related to the eye.

5 patients stopped taking LFG316 because of adverse events:

- 3 patients continued to come to the remaining trial visits
- 2 patients stopped being part of this clinical trial

The percent of patients reporting adverse events during this clinical trial



How many serious adverse events happened?

An adverse event is called serious when it is life-threatening, requires a hospital stay, can cause disability or permanent damage, or can cause a birth defect.

One patient had 3 serious adverse events that were related to the eye**.** These were:

- **Retinal detachment**: The retina pulls away from where it attaches to the eye
- **Proliferative retinopathy:** Damaged and unusual blood vessels around the retina
- **Mycotic endophthalmitis:** A fungal infection that causes swelling of the inside of the eye

The doctors who led this trial do not believe these serious adverse events were caused by the trial drug.

There were no other serious adverse events reported and none of the patients died during this trial.

What were the most common adverse events?

Some patients reported medical problems or adverse events that were not serious. This section reports only the most common adverse events during this clinical trial. Not all adverse events reported are listed here. This summary only lists the adverse events reported by at least 2 of the patients in the trial.

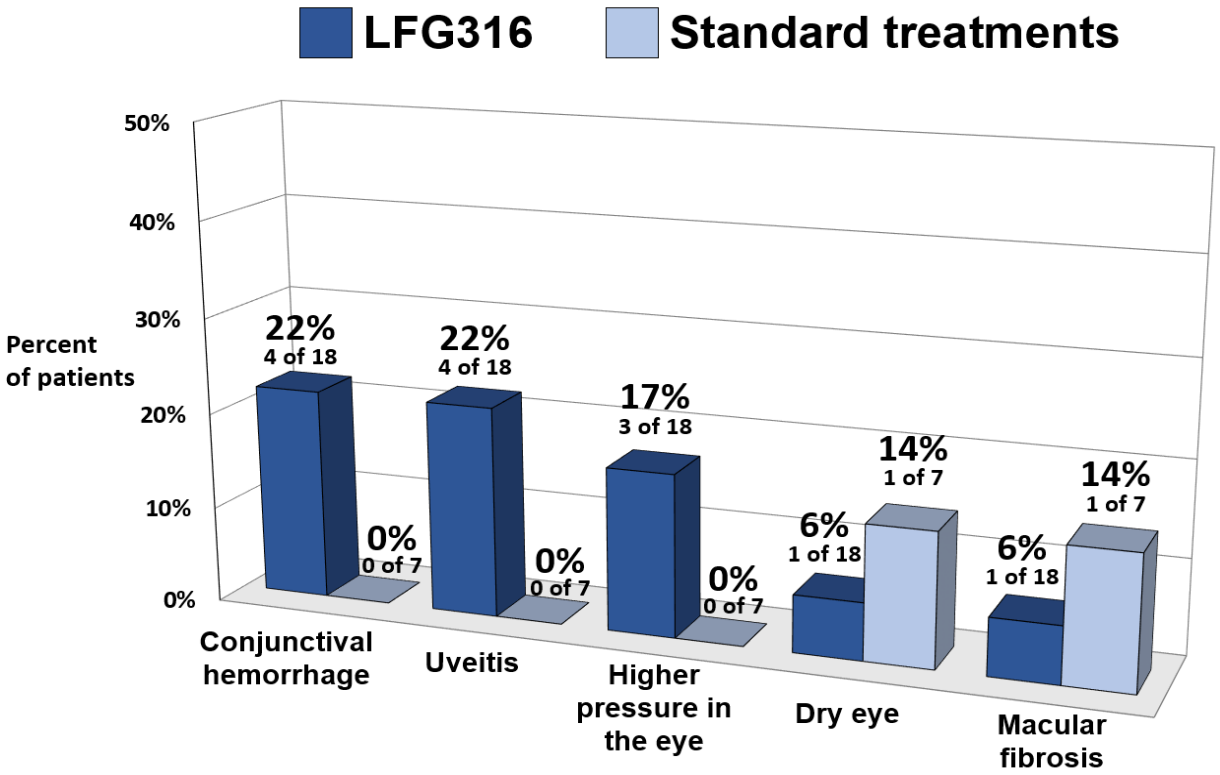
The most common adverse events related to the eye were:

- Conjunctival hemorrhage (spots of blood on the white of the eye)
- Uveitis (that got worse than before)
- Higher pressure in the eye
- Dry eye
- Macular fibrosis (scars on the eye's macula, an area in the center of the retina)

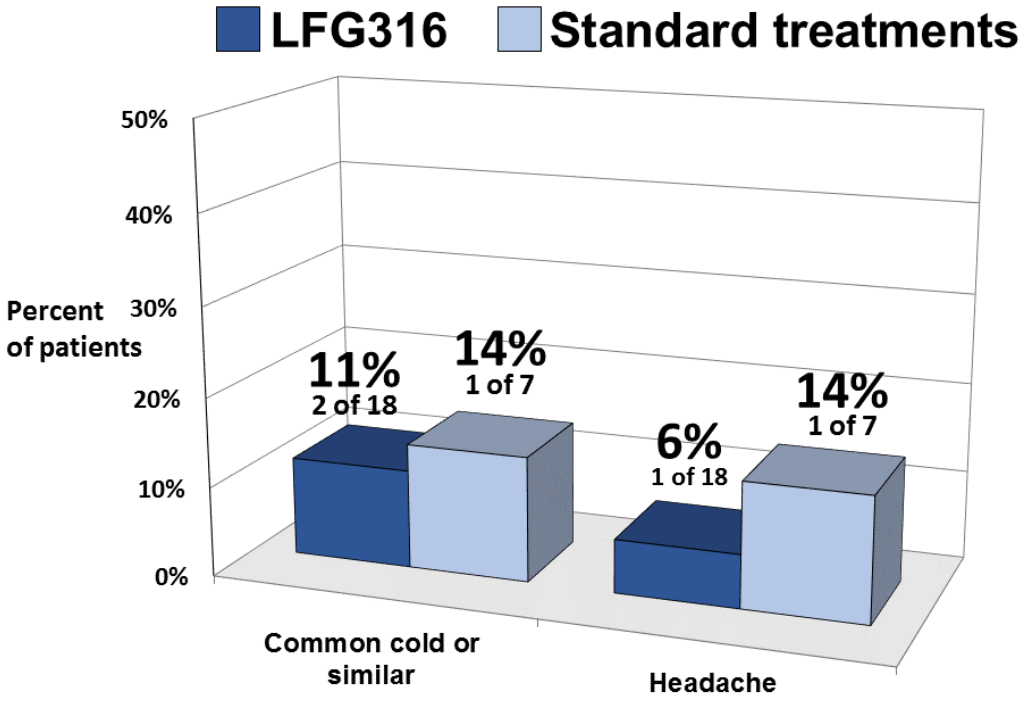
The most common adverse events not related to the eye were:

- Headache
- Common cold or a similar infection

The percent of patients reporting at least 2 adverse events in the eye



The percent of patients reporting at least 2 adverse events not in the eye



For more information about the adverse events that patients reported in this clinical trial, visit novctrd.com. Use clinical trial number CLFG316A2204 to find the scientific summary.

How has this clinical trial helped?

Researchers learned that LFG316 did not affect the symptoms of non-infectious uveitis. The results of this clinical trial helped researchers understand more about LFG316 and the role of the immune system with non-infectious uveitis.

The results presented here are for a single clinical trial. No single clinical trial can 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 patients with non-infectious uveitis. This summary shows only the main results from this trial. Other clinical trials may provide new information or different results.

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

This summary reports on what happened during the first 85 days of this clinical trial. If you were a patient in this clinical trial and have questions about the results, speak with the doctor or staff where you took part in this clinical trial.

This is a summary of the results for one clinical trial.

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. Type **CLFG316A2204** in the search box and click search

This clinical trial was registered on the following websites:

- ClinicalTrials.gov – https://clinicaltrials.gov – National Clinical Trial # NCT01526889
- European Union clinical register – https://www.clinicaltrialsregister.eu/ctr-search – EU Clinical Trial # 2011-003254-90

If more clinical trials are planned, they will appear on the public websites listed above or at www.novartisclinicaltrials.com. When there, search for “LFG316.”

Full trial title: A randomized, active-controlled, open-label, multicenter proof-of-concept study of intravitreal LFG316 in patients with active non-infectious intermediate-, posterior-, or panuveitis requiring systemic immunosuppressive therapy.

Thank you!

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

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