

# Read about this clinical trial's results



## Hello!

Thank you for being in this clinical trial!

You helped doctors learn about a trial medicine called **inclisiran**. The trial medicine is for teenagers with **homozygous familial hypercholesterolemia (HoFH)**. We hope you feel proud of your role!



## What is a clinical trial and why did this trial happen?

A **clinical trial** is research to learn about a medicine. In this trial, doctors wanted to answer these questions about **inclisiran** in teenagers with **HoFH**:

- Did **inclisiran** lower teenagers' "bad" cholesterol from before treatment to 1 year of treatment?
- What medical problems happened to teenagers during this trial?

# What is homozygous familial hypercholesterolemia (HoFH)?

**Homozygous familial hypercholesterolemia (HoFH)** is passed from both parents to their child. It causes high cholesterol. **Cholesterol** is a type of fat in the blood. When cholesterol is high, it can build up in blood vessels and block blood flow. This can cause serious health problems, like heart attacks.



There are 2 main types of cholesterol:

- **LDL cholesterol**, or “bad” cholesterol, which can build up in the blood vessels and cause a heart attack
- **HDL cholesterol**, or “good” cholesterol, which helps the body break down other types of cholesterol and helps protect against heart attacks



## What is inclisiran?



**Inclisiran** is a trial medicine created to lower LDL cholesterol in the blood. You say **inclisiran** as “in-kli-sir-an.”



A **trial medicine** means that doctors are studying it to learn if it works.



Teenagers got **inclisiran** as injections under their skin on their belly.

## Who was in this trial?



13

13 teenagers with **HoFH** were in this trial. All teenagers had **HoFH** and high LDL cholesterol, even though they were taking medicines to lower cholesterol.



12

to 17

years old

They were

12

to 17

years old



4

boys



9

girls

## Where did this trial take place?

The map below shows how many teenagers took part in each country.



# What happened during this trial?



This trial had 2 parts. In **Part 1**, teenagers got one of these:

- 300 milligrams (mg) of the trial medicine called **inclisiran**
- A **placebo**



## What's a placebo?

A placebo has no medicine in it, but looks like the trial medicine. You say placebo as “pluh-see-boh.”

Using a **placebo** helps doctors better understand how **inclisiran** works.



Doctors used a **computer** to assign teenagers by chance to get one of these:



**9 teenagers  
got inclisiran**



**4 teenagers  
got a placebo**

In **Part 1**, the teenagers, their families and the doctors did not know if they were getting **inclisiran** or **placebo**. Teenagers got **inclisiran** or **placebo** 3 times over 1 year.



# What happened during this trial?



Teenagers who completed **Part 1** were in **Part 2**. In **Part 2**, 13 teenagers got **inclisiran**.



They got **inclisiran** as injections up to 3 times over 1 year.



Doctors checked their health for up to 1 month after their last trial visit.

## What was learned from this trial?

### Did inclisiran lower teenagers' "bad" cholesterol from before treatment to 1 year of treatment?

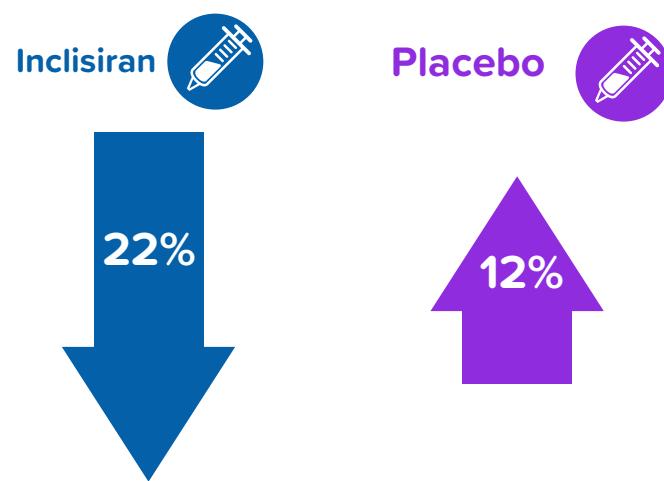


During **Part 1**, doctors measured the level of "bad" or LDL cholesterol in teenagers' blood before and after 1 year of treatment. They compared the change in "bad" cholesterol in teenagers who got **inclisiran** to those who got **placebo**.



**Answer**

Overall, "bad" or LDL cholesterol was lower at 1 year of treatment in teenagers who got **inclisiran** compared to those who got **placebo**.



Teenagers who got **inclisiran** had their cholesterol go down 22% after 1 year of treatment

Teenagers who got **placebo** had their cholesterol go up 12% after 1 year of treatment

## What was learned from this trial?

### What medical problems happened to teenagers during this trial?



Doctors checked teenagers and wrote down all medical problems they had during the trial, even if they were not caused by **inclisiran**.



**Answer**

In **Part 1**, 8 teenagers had medical problems during the trial. No teenagers had serious medical problems.

In **Part 2**, 8 teenagers had medical problems during the trial. No teenagers had serious medical problems.

#### The most common medical problems were:

- COVID-19
- Pain in the upper belly
- Fever
- Flu
- Headache
- Problems where injections were given
- Stomach flu

# Thank you!

Trials like this cannot happen without you and others who join.



Doctors need the results from many trials to find new, possible medicines. We learned many things about **inclsiran** from this trial.



If you would like to **learn more**, ask a parent or another adult, or read the main summary for this trial at: [www.novctrd.com](http://www.novctrd.com)

Search with the trial number  
**CKJX839C12302**



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1-888-669-6682 (US) | +41-61-324 1111 (EU)

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