NOVARTIS

The safety of LNP023 and how the body processes it in people with and without liver disease

Thank you!

Thank you to the participants who took part in the clinical trial. Every participant helped the researchers learn more about **LNP023**, also called iptacopan.

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: CLNP023A2105
Drug studied: LNP023
Sponsor: Novartis

If you were a participant and have any questions about the results, please talk to the doctor or staff at the trial site.

This summary only shows the results of a single clinical trial. Other clinical trials may have different findings.

What was the main purpose of this trial?

The purpose of this trial was to help researchers learn if liver disease changes how the body processes the trial drug LNP023. The researchers also wanted to learn about the safety of LNP023 in people with and without liver disease.

Many health authorities require a trial like this for certain types of drugs before they can approve them. Results from these trials can also change how doctors prescribe the drug for people with liver disease.

LNP023 is a trial drug designed to treat certain health conditions related to the immune system. This trial did not look at the effects of LNP023 on these health conditions.

Liver disease is a group of conditions that cause liver damage and scarring. Over time, this damage and scarring can stop the liver from working well.

Because the liver helps to process certain drugs, liver disease can change how the body processes these drugs.

The main questions this trial was designed to answer:

- Did liver disease change how the body processed LNP023?
- What medical problems did the participants have during this trial?
  Keeping track of the medical problems helped to learn about the safety of LNP023.

Main results: The researchers concluded that the body processed LNP023 about the same in participants with liver disease of any severity compared to healthy participants.

The researchers concluded there were no new safety concerns for LNP023 in this trial.

How long was this trial?

The trial began in November 2021 and ended in June 2022. It was planned for each participant to be in the trial for about 11 days after taking the trial treatment.

The trial staff followed up with participants after 1 month to check for any safety concerns.

Who was in this trial?

38 participants were in this trial – 20 men and 18 women. The participants were 31 to 75 years old. Their average age was 59.

Participants reported their race as:

- White – 36 participants
- American Indian or Alaska Native –1 participant
- Black or African American – 1 participant

Out of the 38 participants:

- 16 participants were considered **healthy** and did not have liver disease
- 22 participants had liver disease and were assigned to 1 of 3 groups based on the severity of their liver disease:
  - **Mild liver disease**
  - **Moderate liver disease**
  - **Severe liver disease**

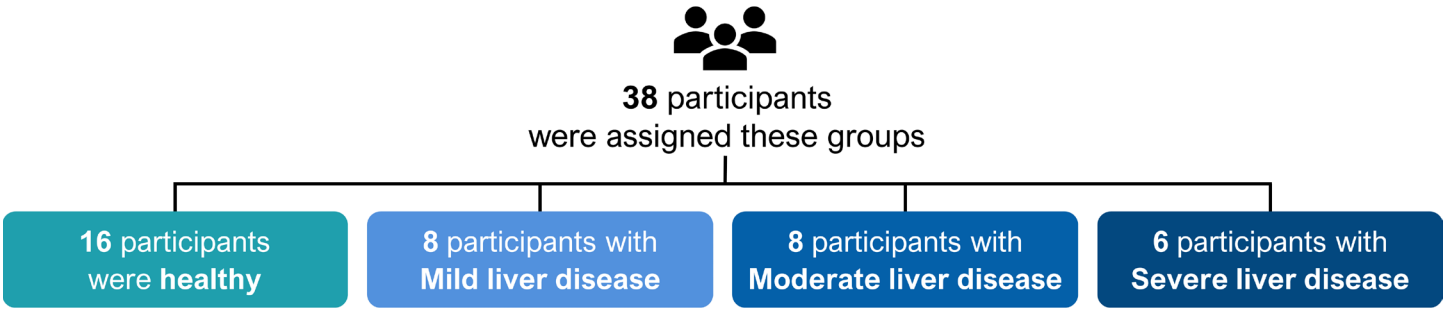
This trial took place in the United States.

For more information, please visit the websites listed at the end of the summary.

What trial treatment did the participants take?

Every participant took 200 milligrams (mg) of **LNP023** one time as a pill.

The participant, trial staff, and researchers knew which trial treatment the participant took. Participants with liver disease continued to take certain medicines for their condition. The graphic below shows how many participants were assigned to each group.



What were the main results of this trial?

Did liver disease change how the body processed LNP023?

No. The researchers concluded that the body processed LNP023 about the same in participants with liver disease of any severity compared to healthy participants.

To find this out, the trial staff took many blood samples from each participant after they took LNP023. The researchers measured the total amount, peak level, time to reach peak level, and how long LNP023 stayed in the participants' blood. Then, they compared these measures in participants with liver disease to healthy participants.

Did liver disease change the total amount of LNP023 in the blood?

No. The total amount of LNP023 in the blood was **about the same** in participants with liver disease of any severity compared to healthy participants.

Did liver disease change the peak level of LNP023 in the blood?

No. The peak level of LNP023 was **about the same** in participants with liver disease of any severity compared to healthy participants. Overall, LNP023 also took about the same length of time to reach the peak level in the blood.

Did liver disease change how long LNP023 stayed in the blood?

No. LNP023 stayed in the blood **slightly longer** in participants with liver disease of any severity compared to healthy participants. However, the researchers concluded this change was not meaningful.

The effect of liver disease on LNP023 in the blood

The chart below summarizes how the body processed LNP023 in participants with liver disease of any severity compared to healthy participants.

For participants with liver disease of any severity compared to healthy participants:

The total amount of **LNP023** was about the **same**	The peak level of **LNP023** was about the **same**	**LNP023** took about the **same** length of time to reach peak level	**LNP023** stayed in the blood a **slightly longer** length of time

What medical problems did the participants have during this trial?

Medical problems that happen during trials are called “adverse events”.

Trial doctors keep track of **all** adverse events that happen in trials, even if they think the adverse events are **not** related to the trial treatments.

Many trials are needed to know if a drug or treatment causes an adverse event.

An adverse event is:

- Any **unwanted sign or symptom** that the participants have during a trial.
- 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.

The adverse events in this section include any that happened up to 30 days after taking the trial treatment.

1 participant (1 of 38 or 3%) with moderate liver disease had adverse events.
This participant had 1 serious adverse event:

- **Bleeding inside the brain from a burst blood vessel** | Basal ganglia hemorrhage

This participant had 1 other adverse event:

- **The lungs could not take in enough oxygen** | Acute respiratory failure

None of the other 37 participants had adverse events. No participants left the trial due to adverse events or died during this trial.

The researchers concluded there were no new safety concerns for LNP023 in this trial.

What was learned from this trial?

This trial helped researchers learn how the body processed LNP023 and its safety in healthy people and people with liver disease.

The researchers concluded that the body processed LNP023 about the same in participants with liver disease of any severity compared to healthy participants. The researchers found no new safety concerns for LNP023 in this trial.

These are the results of a single trial. Other trials may have different results. This was one of many trials a drug goes through. This type of trial helped researchers learn about the safety of a trial drug and if the dose may need to be changed for people with liver disease.

Where can I learn more about this and future trials?

For more information about this trial go to any of the following websites:

- **novctrd.com** – search using the study number **CLNP023A2105**
- **clinicaltrials.gov** – search using the number **NCT05078580**

If more trials are planned, they will appear on the public websites above. When there, search for **LNP023**, **iptacopan**, **liver disease**, or **hepatic impairment**.

Full trial title:

A Phase 1, single dose, open-label study to investigate the pharmacokinetics and safety of iptacopan (LNP023) in participants with mild, moderate, and severe hepatic impairment compared to matched control healthy participants with normal hepatic function

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

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