NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn about the effects of LNP023 in people with immune thrombocytopenia and cold agglutinin disease

Thank you!

Thank you to the participants who took part in the clinical trial for **immune thrombocytopenia (ITP) and cold agglutinin disease (CAD)**. Every participant helped the researchers learn more about the trial drug **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: CLNP023L12201

Novartis drug studied: **LNP023**, also known as **iptacopan**

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.

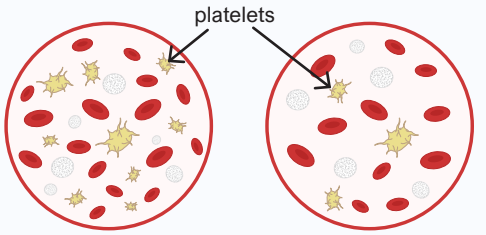
This summary only shows the results of a single clinical trial. Other clinical trials may have different results.

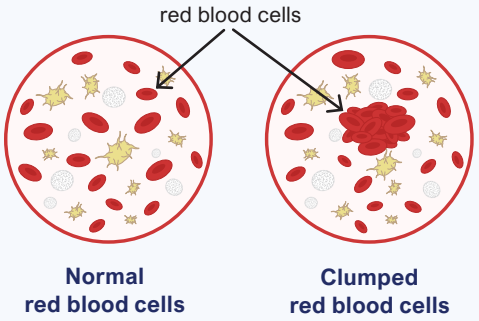
What was the main purpose of this trial?

The main purpose of this trial was to learn about the effects of **LNP023** in people with certain blood disorders caused by the immune system. These disorders are **immune thrombocytopenia (ITP)** and **cold agglutinin disease (CAD)**.

ITP and **CAD** are both types of blood disorders, known as autoimmune hematological disorders. This means they are conditions where the immune system mistakenly attacks healthy blood cells.

In **ITP**, the immune system destroys the platelets. Platelets are important for blood clotting and help stop bleeding. The destruction of platelets can cause bruising, small red or purple spots on the skin, frequent nosebleeds, bleeding in the mouth or gums, or blood in the urine or stool.





In **CAD**, the immune system makes antibodies that attack red blood cells. **Antibodies** are made by our immune system (the body's natural defense) to fight off infections or anything that is harmful to our body.

These antibodies are activated by cold temperatures, causing red blood cells to clump together and break down. This leads to a drop in **hemoglobin**, which is a protein that red blood cells use to carry oxygen throughout the body. A low number of red blood cells (anemia) can cause tiredness, muscle weakness, and pale skin.

Both **ITP** and **CAD** are caused by a part of the immune system in humans called the **complement system** which becomes too active. Current treatments for these conditions do not always work. Even when treatments help at first, the disease can return, or symptoms may get worse. Because of this, researchers are looking for new treatments.

The trial drug **LNP023**, also known as **iptacopan**, works by blocking Factor B, a key protein of the complement system. This may help prevent the destruction of blood cells.

Researchers wanted to see if **LNP023** could help prevent the destruction of platelets in **ITP** and red blood cells in **CAD**.

Drug
iptacopan

Pronounced as
ip-tah-KOH-pan

The trial's purpose was to answer these main questions:

- For participants with **ITP:** Did **LNP023** increase the number of platelets in the blood during the 3 months of treatment?
- For participants with **CAD:** Did **LNP023** increase hemoglobin levels in the blood during the 3 months of treatment?
- What medical problems, also called adverse events, happened during this trial?
  - ↳ An **adverse event** is any sign or symptom that participants have during a trial. Adverse events **may** or **may not** be caused by treatments in the trial.

How long was this trial?

The trial began in December 2021 and ended in May 2024. The trial was designed so that each participant could take part for up to 2 and a half years.

The trial ended earlier than planned due to a business decision. This decision was not due to any safety concerns with **LNP023**.

When the trial ended, researchers created a report of the trial results. This summary is based on that report.

Who was in this trial?

19 participants received treatment in this trial, of whom 9 had **ITP** and 10 had **CAD**. Participants' ages ranged from 20 to 82 years. Their average age was 56 years.

The number of participants by gender and race are shown below.

Gender

- 15 Women
- 4 Men

Race

- 16 White
- 3 Asian

19 participants from **6 countries** received treatment. The map below shows the number of participants who took part in each country.

Participants **could take part** in this trial if they:

- Had **immune thrombocytopenia (ITP)** or **cold agglutinin disease (CAD)**
- Had received at least 1 previous therapy for **ITP** or **CAD**
- Did not have other immune-related conditions

What treatments did the participants receive?

The treatment in this trial was:

LNP023: 200 milligrams (mg), provided as capsules, taken by mouth 2 times a day.

Participants with **ITP** could continue to take some of their regular medicines for **ITP**, along with **LNP023**. However, participants with **CAD** were not allowed to continue their regular medicines for **CAD** along with **LNP023**.

The participants, researchers, and trial staff knew that all participants received **LNP023**.

If a participant's condition got worse or they needed urgent care, the study doctor could provide **rescue therapy**. Rescue therapy or rescue medicines were given to relieve symptoms immediately if participants did not feel relief from their **ITP** or **CAD** symptoms during the trial treatment.

- For participants with **ITP**, rescue therapy included medications like corticosteroids or immunoglobulins, which both help lower the immune response.
- For participants with **CAD**, rescue therapy could include red blood cell transfusions.

Immunoglobulins, also known as **antibodies**, are special proteins made by the immune system to help fight off infections.

A **red blood cell transfusion** is a medical procedure where blood from a donor is given to a patient to replace lost red blood cells and help restore hemoglobin levels.

What happened during this trial?

Before treatment

Up to 2 months

Trial doctors checked the participants' health to make sure they could be in this clinical trial.

During treatment

Around 2 years

The treatment was done in 2 parts. Part A lasted up to 3 months.
Part B lasted up to 2 years.

Part A - 3 months

A total of 19 participants received **LNP023** for 3 months.
They were divided into 2 groups based on their blood disorder.

ITP Group	CAD Group
9 participants	10 participants

Participants who responded to **LNP023** treatment during Part A had the option to continue taking **LNP023** in Part B after a treatment break, which lasted a maximum of 4 weeks.

Some participants who did not have enough improvement in their platelets or hemoglobin levels were still able to continue in Part B if their study doctor thought the treatment was helping them.

Participants who did not continue in Part B stopped treatment and had follow-up visits.

Part B - up to 2 years

A total of 9 participants received **LNP023** during Part B.

ITP Group	CAD Group
1 participant	8 participants

After treatment

Up to 2 months after the last dose

Participants in Part A and B had safety follow-up visits for a month after the last dose of **LNP023**. After another month, they were then contacted by telephone to check for any medical problems.

Trial staff checked the participants' general health through regular tests during the trial.

What were the main results of this trial?

For participants with ITP: Did LNP023 increase the number of platelets in the blood during the 3 months of treatment?

Researchers found that none of the participants with **ITP** had an increase in the number of platelets in their blood during the 3 months of treatment with **LNP023.**

Researchers wanted to find out if participants with **ITP** responded to **LNP023** treatment.

A response meant a participant's number of platelets (platelet count) reached 50,000 per microliter (µL) or higher in their blood, and stayed at that level for at least 2 weeks in a row without needing rescue therapy.

Doctors regularly collected blood samples to check participants' platelet levels.

The results were reported for 8 out of 9 participants who took **LNP023** for 3 months.
One participant did not meet the study requirements and was not analyzed for this result.

For participants with CAD: Did LNP023 increase hemoglobin levels in the blood during the 3 months of treatment?

Researchers found that **5 out of 10 participants (50%)** with **CAD** had improvements in their hemoglobin levels in the blood during the 3 months of **LNP023** treatment.

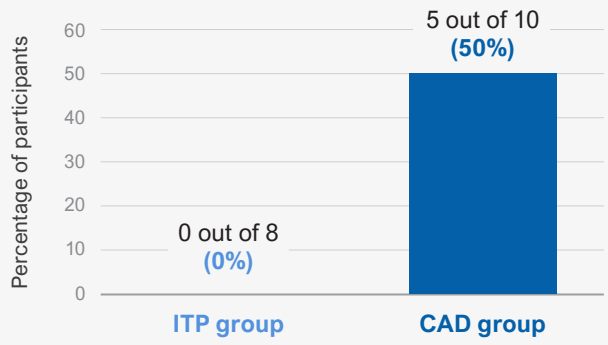
Researchers wanted to find out if participants with **CAD** responded to **LNP023** treatment.

A **response** meant a participant's hemoglobin level in the blood increased by at least 1.5 grams per deciliter (g/dL) from the start of the study and stayed at that level for at least 2 weeks in a row without needing rescue therapy.

Doctors regularly collected blood samples to check participants' hemoglobin levels.

The figure below shows the percentage of participants who responded to **LNP023** treatment.

Percentage of participants who responded to LNP023 treatment



What medical problems, also called adverse events, happened during this trial?

Trial doctors keep track of all **medical problems,** also called **adverse events,** that happen in trials. They track adverse events 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.

This section is a summary of the adverse events that happened from the start of the treatment up to a month after the last dose.

An **adverse event** is:

- Any **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.

A total of **16 out of 19 (84%) participants** had adverse events, including serious and other adverse events.

- 2 participants had adverse events that were considered serious.
- 2 participants left the trial due to an adverse event.
- None of the participants died.

There were no new, unexpected safety concerns with **LNP023**.

How many participants had adverse events?

The table below shows how many participants had adverse events during the treatment.

Summary of adverse events

	ITP group 9 participants	CAD group 10 participants
Overall adverse events **(serious and other adverse events)**	7 of 9 (78%)	9 of 10 (90%)
Had at least **1 serious adverse event**	1 of 9 (11%)	1 of 10 (10%)
Had at least **1 other (not including serious) adverse event**	7 of 9 (78%)	9 of 10 (90%)
Left the trial due to an adverse event	0 of 9 (0%)	2 of 10 (20%)

What serious adverse events did the participants have?

The table below shows the serious adverse events that happened during the trial. A total of 2 participants had serious adverse events, with each participant having 2 events.

Serious adverse events

	ITP group 9 participants	CAD group 10 participants
High levels of creatinine in the blood (a waste product filtered by kidneys) Blood creatinine increased	0 of 9 (0%)	1 of 10 (10%)
Bleeding within the skull Hemorrhage intracranial	1 of 9 (11%)	0 of 10 (0%)
Headache	1 of 9 (11%)	0 of 10 (0%)
Sudden kidney injury Acute kidney injury	0 of 9 (0%)	1 of 10 (10%)

What other (not including serious) adverse events did the participants have?

The table below shows the most common other adverse events that happened during the trial.

Other adverse events

	ITP group 9 participants	CAD group 10 participants
Headache	2 of 9 (22%)	3 of 10 (30%)
Tiny spots under the skin Petechiae	3 of 9 (33%)	0 of 10 (0%)
Weakness Asthenia	0 of 9 (0%)	3 of 10 (30%)
Cough	1 of 9 (11%)	2 of 10 (20%)
Nausea	1 of 9 (11%)	2 of 10 (20%)

What was learned from this trial?

This trial helped researchers learn about the effects of **LNP023** in people with **immune thrombocytopenia (ITP)** and **cold agglutinin disease (CAD).**

The researchers concluded that:

- **LNP023** did not increase the number of platelets in the blood for participants with **ITP**.
- Half of the participants with **CAD** (5 out of 10) responded to **LNP023** treatment as shown by the increase in hemoglobin levels in the blood.
- There were no new, unexpected safety concerns with **LNP023.** The safety results were similar to those seen in previous trials with **LNP023**.

When this summary was written, the sponsor had no plans for future trials of **LNP023** in people with **ITP** and **CAD**.

Where can I learn more about this trial?

More information about the results and adverse events in this trial can be found in the scientific summary of the results available on the Novartis Clinical Trial Results website, www.novctrd.com.

Follow these steps to find the scientific summary:

Go to **www.novctrd.com** → Click **Clinical Trial Results** → Accept the terms **I accept** → Search for **CLNP023L12201**

For more information about this trial, go to any of these websites:

- **clinicaltrials.gov** – search using the number **NCT05086744**
- **clinicaltrialsregister.eu** – search using the number **2021-002039-40**

Other trials with **LNP023** appear on the public websites above. When there, search for **LNP023** or **iptacopan.**

Full clinical trial title: An Open-label, Multi-center, Phase 2 Basket Study to Assess Efficacy, Safety and Pharmacokinetics of Iptacopan (LNP023) in Participants With Autoimmune Benign Hematological Disorders

NOVARTIS

Novartis is a global healthcare company based in Switzerland that provides solutions to address the evolving needs of patients worldwide.

1-888-669-6682 (US); +41-61-324 1111 (EU)
www.novartis.com/clinicaltrials