Clinical Trial a Results Summary

A clinical trial to compare the effects of ETB115 in combination with dexamethasone and dexamethasone alone in people with immune thrombocytopenia

Thank you!

Thank you to the participants who took part in the clinical trial for immune thrombocytopenia. Every participant helped the researchers learn more about the trial drug **ETB115**, also called **eltrombopag**.

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: CETB115JDE01
Drug studied: **ETB115,** also known as eltrombopag
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 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 learn about the effects of **ETB115** in combination with dexamethasone compared to dexamethasone alone in participants with newly diagnosed **immune thrombocytopenia** or **ITP**.

Immune thrombocytopenia (**ITP**) is a condition of low platelet levels in the blood. Low platelet levels mean blood clots are not formed properly, leading to a bleeding disorder. This leads to bruising and bleeding, especially under the skin.

Platelets are tiny cell fragments in the blood that help the blood to clot.

ETB115, also called eltrombopag, is an approved treatment for **ITP**. It works by activating a protein called the thrombopoietin receptor present on the surface of special cells called hematopoietic stem cells in the bone marrow. This helps these special cells grow, divide, and produce more platelets in the body.

Trial drug
ETB115, also called eltrombopag

Pronounced as
el-trom-bo-pag

Dexamethasone is a type of steroid that is widely used to treat many different diseases. It is the most common first choice of treatment for **ITP**. Steroids can help to increase the levels of platelets and reduce bleeding.

In this trial, researchers wanted to learn about the effects of **ETB115** taken with **dexamethasone** for a short duration compared to **dexamethasone** alone for a longer duration.

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The trial purpose was to answer these main questions:

- How many participants continued to show a **response** 1 year after starting **ETB115** treatment in combination with **dexamethasone** compared to **dexamethasone** alone?

  ↳ **Response** to treatment means maintaining a particular blood platelet levels after stopping the treatment, without any moderate to severe bleeding events.

- What adverse events did the participants have during this trial?

  ↳ An **adverse event** is any sign or symptom that participants have during a trial.

How long was this trial?

The trial began in October 2020 and ended in September 2023. The participants could be in this trial for about 1 year.

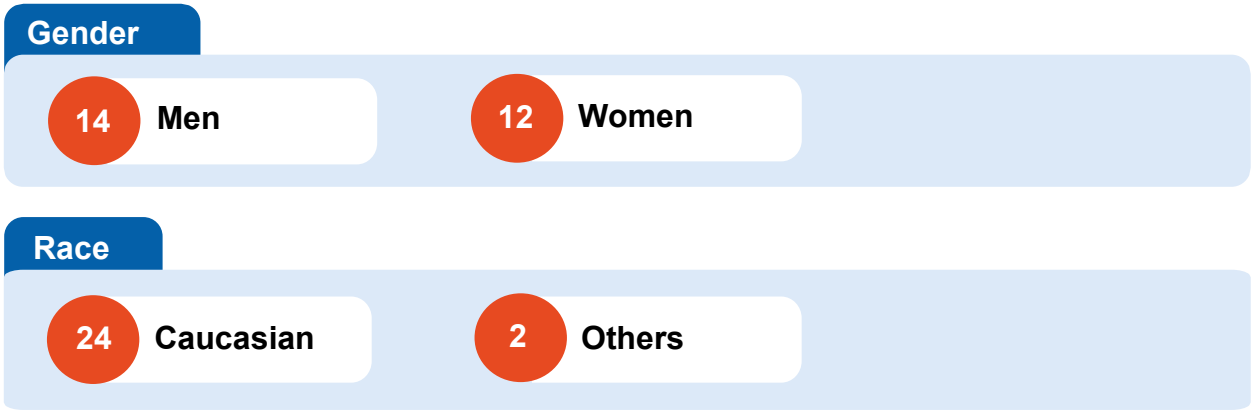
The researchers completed this trial with fewer participants than initially planned. The decision to conduct this trial with fewer participants was because of slow enrollment and not because of any safety concerns.

When the trial ended, the researchers collected information from participants and created a report of the trial results. This summary is based on that report.

Who was in this trial?

26 participants with **immune thrombocytopenia (ITP)** entered this trial. Participants' ages ranged from 27 to 80 years. Their average age was 53 years.

All the participants were from Germany. The number of participants by gender and race are shown below.



The participants could take part in this trial if they:

- were 18 years of age or older
- were diagnosed with **ITP** within 3 months of joining the trial
- had low levels of platelets that needed treatment
- were not previously treated for **ITP**
- did not have bleeding problems that were life-threatening

What treatments did the participants receive?

The treatments in this trial were:

ETB115: Participants took a tablet by mouth once a day during this trial.

The starting dose of **ETB115** was 50 milligrams (mg) once a day. Participants could take up to 75 mg of **ETB115** once a day during this trial based on their platelet levels.

Dexamethasone: Participants took a 40 mg tablet by mouth once a day for the first 4 days of a treatment cycle. In this trial, the treatment cycle lasted 14 to 28 days.

A **treatment cycle** is a treatment period that can be repeated after a fixed number of days.

In this trial, the participants, researchers, and trial staff knew what treatment each participant took.

What happened during this trial?

Before treatment — Up to 14 days

Trial doctors checked the participants' **ITP** and overall health to ensure they could take part in this clinical trial.

During treatment — Up to 1 year

Participants were put into one of the following groups randomly. Neither participants nor their doctors were able to choose which group they were in.

Group 1: 13 participants
Participants took an **ETB115** tablet once a day for about 6 months with 1 cycle of **dexamethasone** tablets for 4 days.

Group 2: 13 participants
Participants took **dexamethasone** tablets alone for up to 3 cycles of 14 to 28 days each.

Trial doctors could increase or decrease the dose of **ETB115** or stop **ETB115** or **dexamethasone** altogether depending on participants' platelet levels and their response to the treatment.

Some participants could continue their assigned treatment or switch between the treatments based on their platelet levels until 1 year during the trial.

After treatment — 1 month after the last dose

Participants returned to their trial site and trial doctors checked participants for their overall health.

What was the main result of this trial?

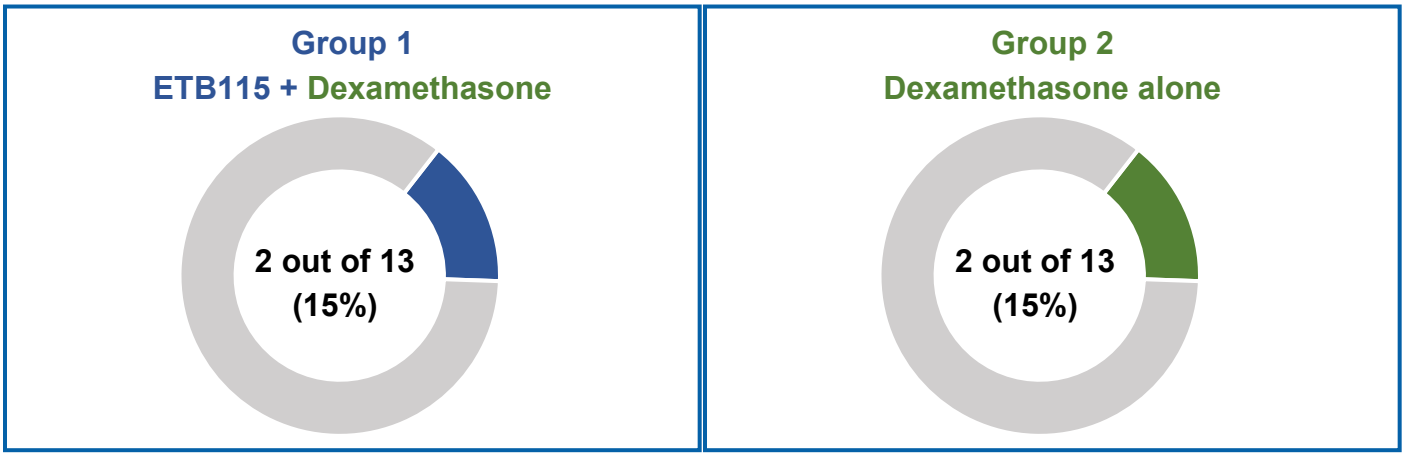
How many participants continued to show a response* 1 year after starting ETB115 treatment in combination with dexamethasone compared to dexamethasone alone?

The same number of participants in both **Group 1** and **Group 2** continued to show a response 1 year after starting the treatment in this trial.

To learn this, researchers monitored platelet levels through blood tests and assessed bleeding events in participants during the trial.

***Response** to treatment means maintaining a particular blood platelet levels after stopping the treatment, without any moderate to severe bleeding events.

Number of participants (percentage) who showed a response after 1 year of starting treatment



What adverse events did the participants have?

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.

This section is a summary of the adverse events that happened from the start of treatment until 1 month after participants left or completed the trial.

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.

1 of 26 participants did not receive any treatment during the trial. Therefore, this summary presents the results of 25 participants in this section.

Almost all the participants (24 of 25) had adverse events. 10 participants had adverse events that were considered serious. 1 participant died during the trial. 2 participants left the trial due to an adverse event. The researchers concluded there were no new safety concerns for **ETB115** for this trial.

How many participants had adverse events?

Participants who:	Group 1 ETB115 + Dexamethasone 12 participants	Group 2 Dexamethasone alone 13 participants
Had at least 1 serious adverse event	**5 of 12** 42%	**5 of 13** 39%
Had at least 1 other adverse event	**11 of 12** 92%	**13 of 13** 100%
Left the trial due to an adverse event	**2 of 12** 17%	**0**
Died during the trial	**0**	**1 of 13** 8%

What serious adverse events did the participants have?

The table below shows the most common serious adverse events that happened in 2 or more participants in any group.

	Group 1 ETB115 + Dexamethasone 12 participants	Group 2 Dexamethasone alone 13 participants
Worsening of immune thrombocytopenia (ITP)	**2 of 12** 17%	**0**
Blood clot in a blood vessel of the lungs Pulmonary embolism	**0**	**2 of 13** 15%

What other adverse events did the participants have?

The table below shows the other adverse events that happened in 20% or more participants in any group.

	Group 1 ETB115 + Dexamethasone 12 participants	Group 2 Dexamethasone alone 13 participants
Constipation	**3 of 12** 25%	**0**
COVID-19 infection	**3 of 12** 25%	**4 of 13** 31%
Diarrhoea	**3 of 12** 25%	**3 of 13** 23%
Fever Pyrexia	**1 of 12** 8%	**4 of 13** 31%
Headache	**2 of 12** 17%	**4 of 13** 31%
Pain in the abdomen Abdominal Pain	**3 of 12** 25%	**0**
Rash	**1 of 12** 8%	**3 of 13** 23%
Tiredness Fatigue	**2 of 12** 17%	**3 of 13** 23%

What was learned from this trial?

Researchers learned about the effects of **ETB115** in combination with **dexamethasone** compared to **dexamethasone** alone in people with **immune thrombocytopenia or ITP**.

The researchers concluded that there was no difference in the continued response 1 year after starting treatment with **ETB115** in combination with **dexamethasone** compared to **dexamethasone** alone. However, the trial ended with fewer participants than initially planned.

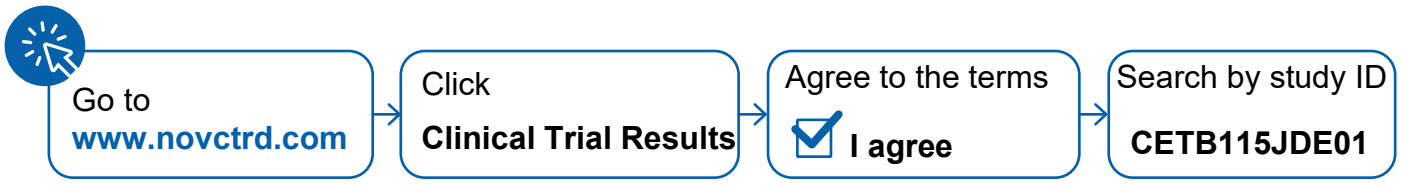
The researchers did not find new safety concerns for **ETB115** in this trial.

When this summary was written, there were no plans for future trials with **ETB115** in people with **ITP**. If more trials are planned for **ETB115** for participants with **ITP**, they will appear on the public websites below.

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:



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

- www.clinicasltrials.gov - search using the number **NCT04346654**
- clinicaltrialsregister.eu - search using the number **2019-002658-21**

If more trials are planned, they will appear on the public websites above.

When there, search for ETB115, or eltrombopag.

Full clinical trial title: A Phase II, randomized (1:1) open label study to assess the efficacy and safety of eltrombopag in combination with dexamethasone compared to dexamethasone, as first-line treatment in adult patients with newly diagnosed immune thrombocytopenia (XPAG-ITP)

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