NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn more about the effects of ETB115 in people with severe aplastic anemia

Thank you!

Thank you to the participants who took part in the clinical trial for **severe aplastic anemia (SAA)**. 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: CETB115E2202

Novartis 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 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 purpose of this trial was to learn more about the effects of **ETB115** in participants with **refractory or relapsed severe aplastic anemia (SAA)** in China. **Refractory** means previous treatment didn't work. **Relapsed** means that SAA came back after treatment.

Severe aplastic anemia (SAA) is a disease in which bone marrow does not make enough blood cells. Bone marrow is the tissue inside of bones that helps make:

- **Red blood cells**, which carry oxygen
- **White blood cells**, which are part of the immune system and help the body fight infection and other diseases
- **Platelets**, which help blood to clot and stop bleeding

People with SAA often feel weak and tired, get more infections, and have bruising and bleeding that can be hard to stop. They may have **blood transfusions** to replace red blood cells and platelets their bone marrow isn't making.

ETB115 also called eltrombopag is a drug designed to signal bone marrow to make more blood cells. When the trial was planned, **ETB115** was approved to treat certain people with SAA in some countries, such as the United States and European Union. However, it had not been approved in China to treat people with SAA.

Trial drug
ETB115 also called eltrombopag

Pronounced as
el-**traam**-buh-pag

The trial purpose was to answer these main questions:

- How many participants had their blood cell counts go up and needed fewer transfusions within 6 months of taking ETB115?
- What adverse events did the participants have?
  - ↳ An **adverse event** is any sign or symptom that participants have during a trial.

How long was this trial?

The trial began in December 2019 and ended in May 2023. Participants were in the trial for up to about 3 years.

Who was in this trial?

20 participants with **severe aplastic anemia (SAA)** received treatment in this trial – 15 men and 5 women.

Participants' ages ranged from 18 to 69 years. Their average age was 39 years.

The number of participants by race is shown below.

Race

20	Asian, Chinese

The participants could take part in this trial if they:

- Had taken one or more past treatments for SAA that did not work to raise blood cell counts or SAA came back after treatment
- Could not receive a stem cell transplant, which is a treatment that replaces a person's bone marrow with healthy cells
- Did not have other blood-related health conditions

This trial took place in China.

What treatments did the participants receive?

The treatment in this trial was:

ETB115, also known as eltrombopag: 25 to 150 milligrams (mg) taken by mouth as tablets each day. Each participant's dose started at 25 mg per day. Then, their dose could slowly go up to 150 mg per day.

Along with the treatment above, participants could take other treatments for SAA if needed, such as transfusions.

In this trial, the participants and clinical trial team knew what treatment each participant took. All participants took **ETB115**.

What happened during this trial?

Before treatment — 1 month

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

During treatment — Up to about 3 years

20 participants took **ETB115** by mouth as tablets for at least 6 months:

- Participants started at 25 milligrams (mg) per day
- Then, their dose could slowly go up to 150 mg per day

If the participant's blood cell counts went up enough at 6 months, they could continue taking **ETB115** until:

- The trial ended, or
- **ETB115** was approved for doctors to prescribe for SAA in China

Researchers checked the participants' SAA and general health throughout the trial.

After treatment — 1 month

Participants returned to their trial site one time after receiving their last dose of treatment for a follow-up visit to check their health.

What were the main results of this trial?

How many participants had their blood cell counts go up and needed fewer transfusions within 6 months of taking ETB115?

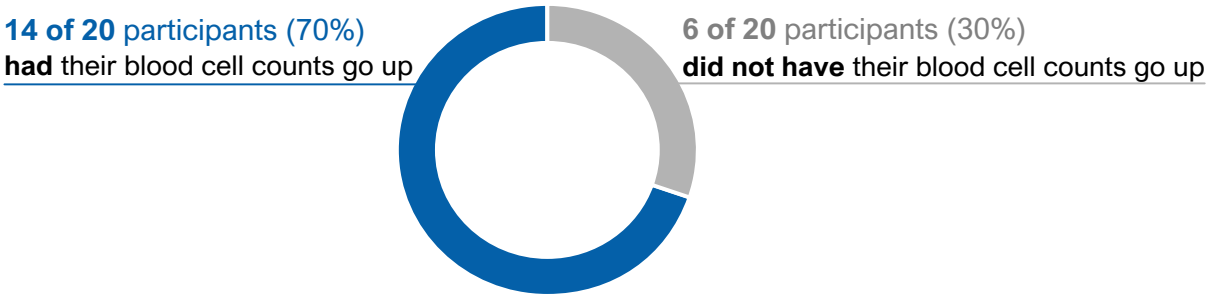
14 of the 20 participants (70%) had their blood cell counts go up and needed fewer transfusions within 6 months of taking **ETB115**.

To learn this, the trial staff measured the participants' red blood cell, white blood cell, and platelet counts. The trial staff kept track of how many participants had higher counts of at least one type of blood cell within 6 months. Trial staff also kept track of the number of transfusions participants needed.

The participants' blood cell count was considered higher if the participant had **at least one** of these changes:

- **Platelet count** went up by 20,000 cells per microliter (μL) of blood, or did not need a platelet transfusion for at least 2 months
- **Hemoglobin** (a measure of red blood cells) went up by 15 grams per liter (g/L) of blood, or needed fewer red blood cell transfusions
- **Neutrophils** (a measure of white blood cells) went up by 500 cells per microliter (μL) of blood, or doubled if the number before treatment was very low

Number of participants whose blood cell counts went up



What were the other results of this trial?

How many participants had their blood cell counts stay up and needed fewer transfusions after 12 months of taking ETB115?

13 of the 20 participants (65%) had their blood cell counts stay up and needed fewer transfusions at 12 months of taking **ETB115**. Some participants also went longer without red blood cell or platelet transfusions while taking **ETB115**.

The researchers concluded that overall, participants received fewer blood cell transfusions while taking **ETB115** for up to a year.

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 trial treatment to 30 days after the last dose of treatment.

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.

All 20 participants had adverse events. 8 participants had adverse events that were considered serious. No participants died during the trial or left the trial due to an adverse event. The researchers concluded there were no new safety concerns for **ETB115** in this trial.

How many participants had adverse events?

Participants who:	ETB115 20 participants
Had at least 1 **serious adverse event**	8 of 20
Had at least 1 **other adverse event**	19 of 20
Left the trial due to an adverse event	0 of 20
Died during the trial	0 of 20

What serious adverse events did the participants have?

8 participants had serious adverse events. No participants died.

The table below shows the most common serious adverse events that happened in **2 or more** participants (10% or more).

	ETB115 20 participants
Lung infection Pneumonia	3 of 20 15%
Medicine dosing error Intentional product misuse	2 of 20 10%
Infection in the nose, throat, and airway Upper respiratory tract infection	2 of 20 10%

What other adverse events did the participants have?

19 participants had other adverse events.

The table below shows the other adverse events that happened in **5 or more** participants (25% or more).

	ETB115 20 participants
Infection in the nose, throat, and airways Upper respiratory tract infection	**8 of 20** 40%
High blood levels of the waste product creatinine Blood creatinine increased	**6 of 20** 30%
Fever Pyrexia	**5 of 20** 25%
High blood levels of the waste product bilirubin Hyperbilirubinemia	**5 of 20** 25%
High blood levels of the waste product uric acid Hyperuricemia	**5 of 20** 25%

What was learned from this trial?

Researchers learned about the effects of **ETB115** in people with **severe aplastic anemia (SAA)** in China. These results were similar to results from trials of **ETB115** in different countries.

The researchers concluded that:

- Participants had their blood cell counts go up and needed fewer transfusions within 6 months of taking **ETB115**
- Participants' blood cell counts tended to go up during the trial, and some participants went a little more than a year without a transfusion while taking **ETB115**
- There were no new safety concerns for **ETB115** in this trial

In February 2023, **ETB115**, also known as eltrombopag, was approved in China for people with relapsed or refractory SAA based on results from this and other trials.

When this summary was written, there were no plans for future trials of **ETB115** in people with relapsed or refractory SAA.

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 this website:

- **clinicaltrials.gov** – search using the number **NCT03988608**

Other trials of **ETB115** may also appear on the public websites above. When there, search for **ETB115** or **eltrombopag**.

Full clinical trial title: A Non-randomized, Open-label, Multi-center, Phase II Study to Assess the Safety and Efficacy of Eltrombopag in Chinese Subjects With Refractory or Relapsed Severe Aplastic Anemia

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