

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 **ETB115**. The trial medicine is for people with **severe aplastic anemia (SAA)**. 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 trial medicine. In this trial, doctors wanted to answer these questions about **ETB115** in people with **SAA**:

- How many people had all 3 of their blood cell counts go up?
- What medical problems happened to people during this trial?

What is severe aplastic anemia (SAA)?

SAA is a disease in which bone marrow does not make enough blood cells. It can affect children and adults.



What are bone marrow and blood cells?

Bone marrow is the tissue inside of bones that helps make:

- **Red blood cells**, which carry oxygen in the blood to all parts of the body
- **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 sick more often, get bruises more easily, and have bleeding that can be hard to stop.



What is ETB115?



ETB115 is a trial medicine created to treat **SAA** by signaling for the bone marrow to make more blood cells.



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



People took **ETB115** by mouth as a tablet each day.

Who was in this trial?



36

people were in this trial. Everyone had **SAA** and had not been treated for it before.



They were
9 to 72
years old



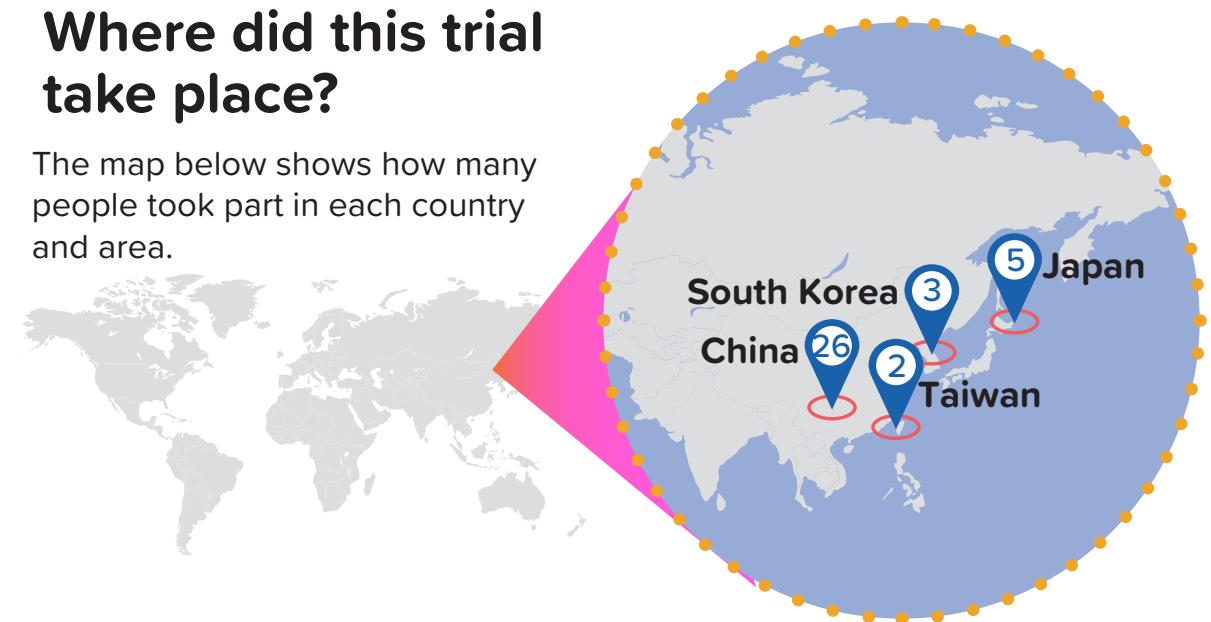
15
males



21
females

Where did this trial take place?

The map below shows how many people took part in each country and area.



What happened during this trial?



Everyone got all of these:

- The trial medicine called **ETB115**
- The **usual medicines** for **SAA** called **cyclosporine** and **r-ATG (rabbit anti-thymocyte globulin)**



What are **usual medicines** for **SAA**?



The usual medicines are what doctors currently use to treat **SAA**.



People took cyclosporine by mouth as liquid or capsules



People got r-ATG through a vein called an IV infusion



Everyone took **ETB115** for up to 1 year and the **usual medicines** for up to 2 years.



Doctors checked their health for about 2 years after they stopped taking **ETB115**.

What was learned from this trial?



How many people had all 3 of their blood cell counts go up after 6 months of taking **ETB115** with the **usual medicines**?



Trial staff took blood from people during the trial. They measured people's blood for the number of:

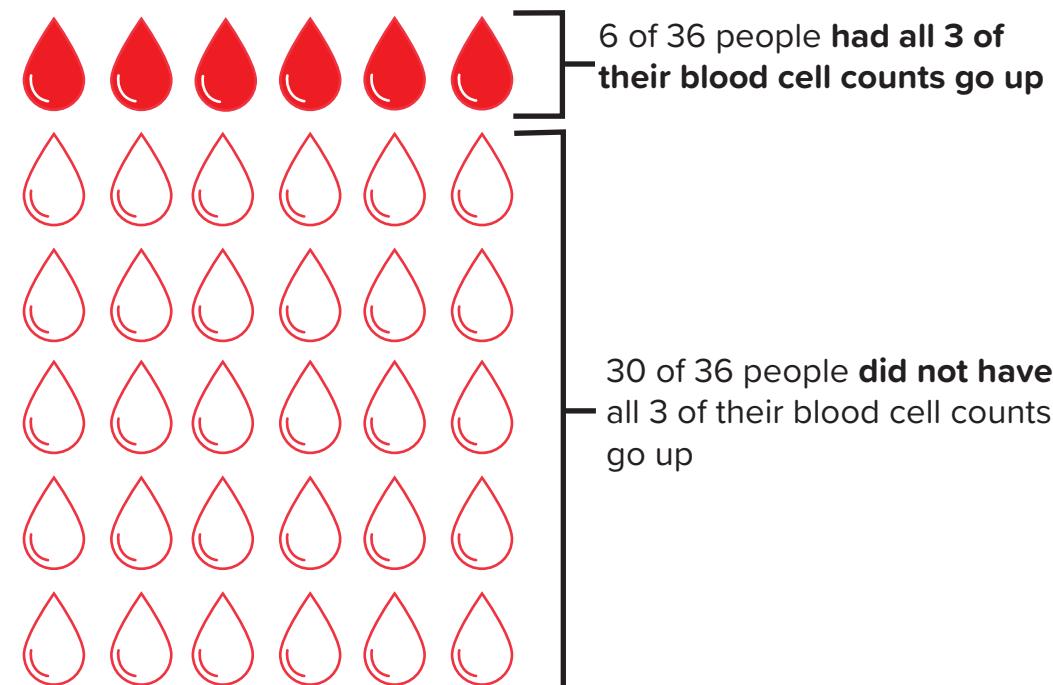
- Red blood cells
- White blood cells
- Platelets

Trial staff measured the blood before people started taking **ETB115** and many times while taking it. They counted how many people had all 3 of their blood cell counts go up after 6 months.



Answer

6 of 36 people (17%) had all 3 of their blood cell counts go up after 6 months of taking **ETB115** with usual medicines.



What else was learned?



How many people had their blood cell counts go up after 1 year of taking **ETB115**?



Answer

After 1 year of taking **ETB115**:

- 11 people had all 3 of their blood cell counts go up
- 24 people had at least 2 of their blood cell counts go up

What else was learned?



What medical problems happened to people during this trial?



Doctors checked people and wrote down all medical problems they had during the trial, even if they were not caused by **ETB115** or the **usual medicines**.



Answer

Everyone had medical problems during this trial. 14 people had serious medical problems.

The most common serious medical problems were:



Blood test showed too much uric acid in blood



Serious complication of an infection



Blood test showed not enough potassium in blood

Other common medical problems were:

- Blood test showed not enough potassium in blood
- Blood test showed too much uric acid in blood
- Blood test showed possible sign of liver damage
- Blood sugar went up
- Fever

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 **ETB115** 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

Search with the trial number
CETB115G2201



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