

Graphic

clinical trial results summary



Hello!

Thank you for taking part in this clinical trial!

Because of your help, we learned more about a trial drug called **ACZ885** for people with **sickle cell anemia**. We hope you feel proud of your role helping to find a treatment for sickle cell anemia.



Main questions

1

Did **ACZ885** lower people's pain from **sickle cell anemia**?

2

What **medical problems** happened during this trial?



Answers

No, the researchers decided that **ACZ885** did not lower people's pain in this trial. Overall, everyone in the trial had slightly less pain. People who got ACZ885 had about the same amount of pain as people who did not get ACZ885.

Most people had medical problems during this trial – some were serious. Many were related to **sickle cell anemia**, such as **pain from a sickle cell crisis**.

Who was in this trial?



➡ ○ **start here!**

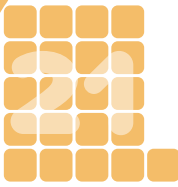


took part in this trial

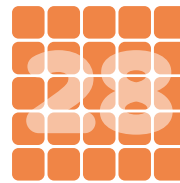
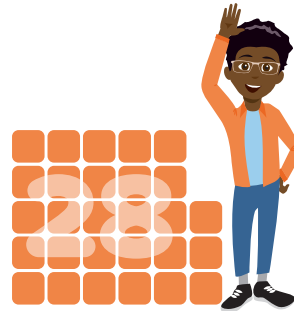
They were
8 to 20
years old



21
females



28
males



They went to trial sites in:



- Canada
- Germany
- Israel
- South Africa
- Turkey
- United Kingdom
- United States

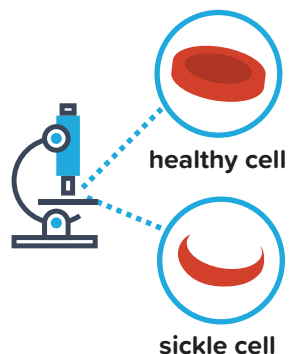


Everyone had **sickle cell anemia**



What is **sickle cell anemia**?

Sickle cell anemia is a health problem caused by sickle cells, which are blood cells with an unusual shape.



These sickle cells break down faster and cause **inflammation**, or swelling, in blood vessels. Inflammation is how the body responds to things that could harm it. Too much inflammation can cause pain.



normal blood vessel
with **sickle cells**

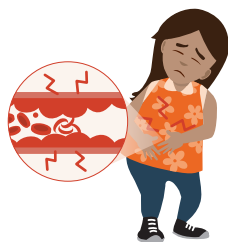


blood vessel
with **inflammation**

Sickle cells can get stuck in swollen blood vessels. If too many get stuck, they can block the vessel.



blocked
blood vessel

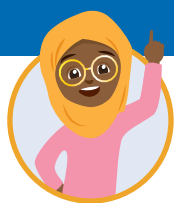


sickle cell crisis

Scientists think **inflammation** and **blocked** blood vessels can cause a **sickle cell crisis** which is very **painful**.

next page!

What happened during this trial?



➡ ○ **start here!**



Researchers used a **computer** to randomly assign people their trial treatment:



25 people
got **ACZ885**

24 people
got a **placebo**



What is **ACZ885**?

ACZ885 is a trial drug that researchers think might lower the pain from **sickle cell anemia**. This trial was designed to find out if it could.



What is a placebo?

The **placebo** (pluh-see-bo) had **no trial drug** in it. This means they did not get ACZ885.

Using a placebo helps researchers better understand if ACZ885 works.

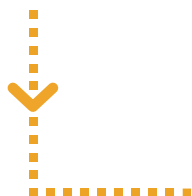
Part 1



Part 1 of this trial lasted for **24 weeks**



Everyone got their trial treatment as **shots** every **4 weeks**



Researchers asked everyone if they wanted to continue to **Part 2**. This was set up the same as **Part 1**, except everyone got **ACZ885**. This helped researchers learn even more.



Part 2



Part 2 of this trial lasted for **24 weeks**



This trial **ended early** because the researchers learned enough to answer their questions.

next page!



What was learned from this trial?

1 Did **ACZ885** lower people's pain from **sickle cell anemia**?



To answer this question, everyone in the trial reported how much pain they felt each day during their treatment. If people reported **less pain**, it might be a sign that their treatment lowered their pain.



Researchers compared the pain reported

from



people who got **ACZ885**

with



people who got the **placebo**



No, the researchers decided that **ACZ885** did not lower people's pain in this trial. Overall, everyone in the trial had slightly less pain. People who got ACZ885 had about the same amount of pain as people who did not get ACZ885.

2 What **medical problems** happened during this trial?



To answer this question, the trial doctors checked for medical problems during the trial and people also reported medical problems. Researchers track **all** medical problems, even if they aren't related to the trial drug.

The most common **serious medical problem** was:



pain from a sickle cell crisis

Other **medical problems** were:



pain



headache



cold or flu



Most people had **medical problems** during this trial – some were serious. Many of these were related to **sickle cell anemia**, such as **pain from a sickle cell crisis**.



What else was learned in this trial?

Did the people who got **ACZ885** in this trial have:



... **Lower** signs of inflammation?

The people who got **ACZ885** had lower signs of inflammation in their blood compared to those who didn't.



... **Fewer** missed days of school or work?

The people who got **ACZ885** may have started to miss fewer days of school or work towards the end of their treatment.



... **Fewer** blood transfusions?

The people who got **ACZ885** had fewer **rescue blood transfusions** on average compared to those who didn't. But, there wasn't much difference in how many **overall** blood transfusions they had to get.



What is a rescue blood transfusion?

A kind of transfusion that people with sickle cell anemia may get when their symptoms suddenly get worse.



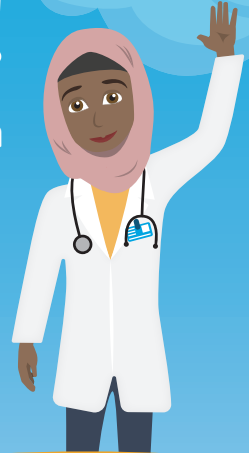
... **Less** signs of the breakdown of red blood cells?

The people who got **ACZ885** had no difference in their signs of the breakdown of red blood cells compared to those who didn't.



Thank you!

Trials like this cannot happen
without the participants.



Researchers need the results from many trials to find new, possible treatments. Although **ACZ885** didn't lower people's pain, we still learned many things. This trial is one of many steps on the path to find new and better ways to treat sickle cell anemia.



If you would like to **learn more**, please read the main summary for this trial.

