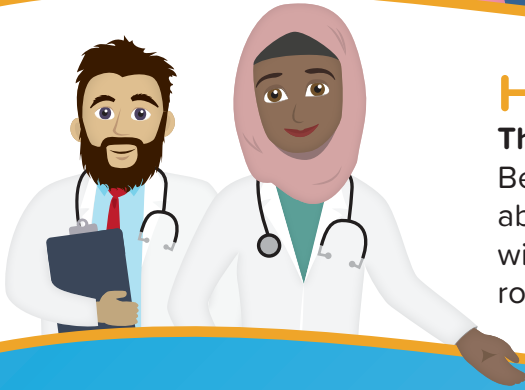


clinical trial results

graphic summary



Hello!

Thank you for taking part in this clinical trial!

Because of your help, we learned more about a trial drug called **CDZ173** for people with **APDS**. We hope you feel proud of your role helping to find a treatment for APDS.



Main questions

- 1 Which amount of **CDZ173** lowered the protein, **PI3K delta**, the most?
- 2 Did **CDZ173** change some symptoms of **APDS**?
- 3 What medical problems happened during this trial?



Answers

The high amount of CDZ173 lowered PI3K delta the most.

Yes, people who took CDZ173 had less swelling in **lymph nodes** and a higher number of certain **B cells**.

Most people had medical problems during this trial. Some were serious. Researchers decided CDZ173 was safe for the people in this trial.



Turn the page to learn about PI3K delta, lymph nodes, and B cells!

What is APDS and PI3K delta?



APDS is a health problem caused by an overactive protein in the body's **immune system**. This protein is called **PI3K delta**.



What's the **immune system**?

The immune system is made of cells and proteins that protect the body from infections, like a cold or the flu.



PI3K delta controls how B cells grow and protect the body. **B cells** are some of the main cells in the immune system.

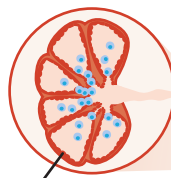


For someone with **APDS**, **PI3K delta** is overactive. This causes B cells to die early and not work as well to protect the body.



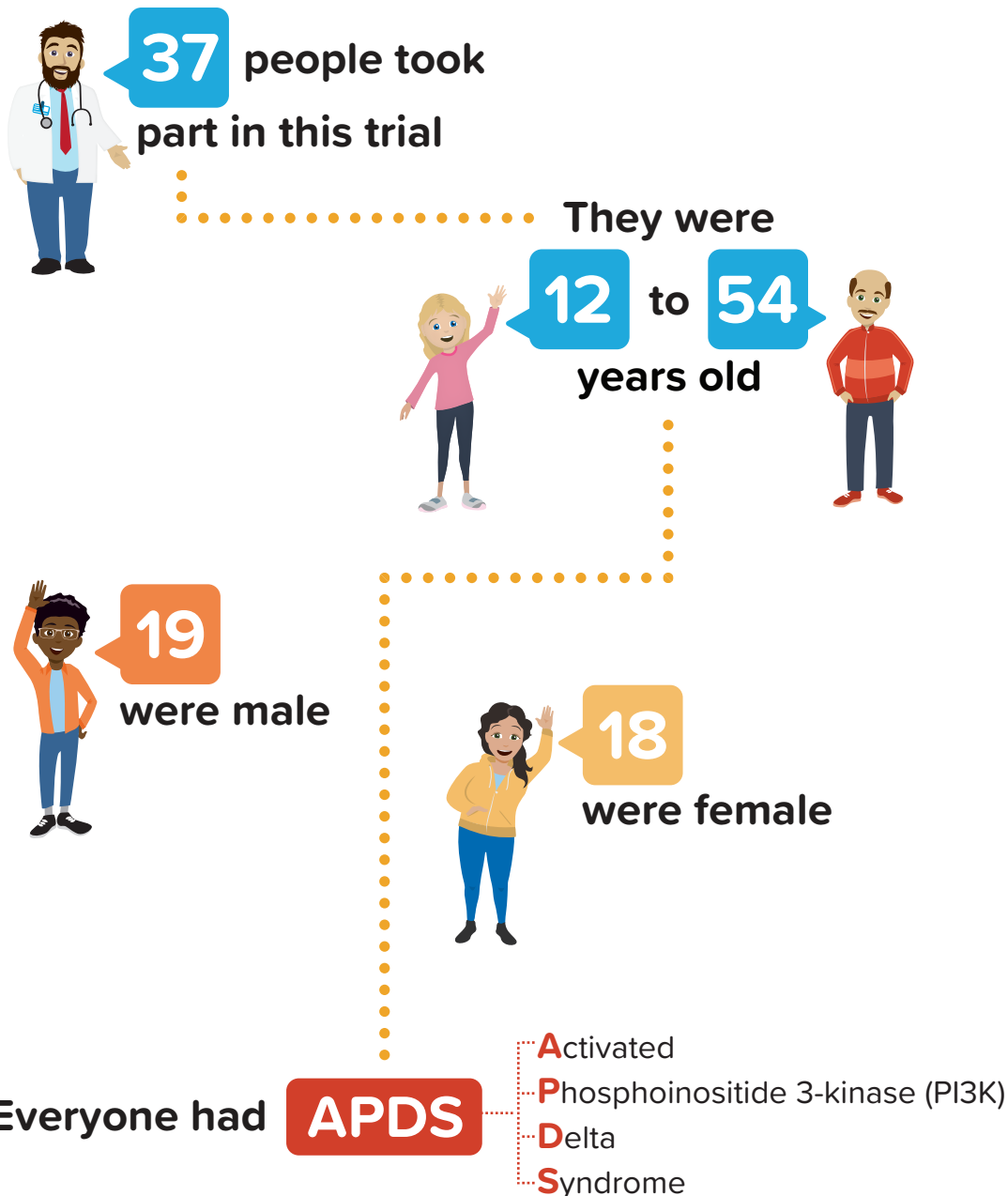
What are the **symptoms** of **APDS**?

People with **APDS** have infections more often that can be more serious. This is because they have fewer B cells to protect their body.



APDS can also cause swollen and painful lymph nodes. **Lymph nodes** are tiny, bean-shaped organs that filter out infections from the body.

Who was in this trial?



Where did this trial happen?

People went to trial sites in:



- Belarus
- Czech Republic
- Germany
- Ireland
- Italy
- Netherlands
- Russia
- United Kingdom
- United States

What happened during Part 1?

Part 1



The people in this part helped researchers learn which **dose** of **CDZ173** lowered **PI3K delta** the most. This helped researchers decide what dose to give in **Part 2**.



What's **CDZ173**?

CDZ173 is a trial drug designed to stop overactive **PI3K delta**.



6 people took **CDZ173**




What's a **dose**?

The dose is the amount of the trial drug people took.




1st

they took the
 **Low dose**
for **4 weeks**




2nd

they took the
 **Medium dose**
for **4 weeks**



3rd

they took the
 **High dose**
for **4 weeks**



Researchers checked for any safety concerns while people took each dose. They looked at the results from each dose and decided to use the **High dose** in **Part 2**.

What happened during Part 2?

Part 2



The people in this part helped researchers learn if **CDZ173** changed some symptoms of **APDS**.

Some people took the **high dose of CDZ173** and others took a **placebo** (pluh-see-bo).



What's a **placebo**?

A **placebo** has **no trial drug** in it.
This means they did not get CDZ173.
Using a placebo helps researchers better understand if CDZ173 works.



Researchers used a **computer** to randomly assign people to take either treatment:

 **CDZ173** <... or ...>  **placebo**

21 people
took **CDZ173**



10 people
took a **placebo**



People took their
treatment for
12 weeks



What was learned from this trial?

1 Which dose of **CDZ173** lowered **PI3K delta** the most?

To answer this question, the trial doctors took blood samples from everyone in Part 1 after each dose of CDZ173. They compared these samples to find out which dose lowered PI3K delta the most.

Researchers compared blood samples after people took:



Low dose



Medium dose



High dose



Answer

The high dose of CDZ173 lowered PI3K delta the most and for the longest time.

2 Did **CDZ173** change some symptoms of **APDS**?

To answer this question, the trial doctors looked at swelling in people's lymph nodes and the number of certain B cells in their blood samples.

Researchers compared the swelling in lymph nodes and number of certain B cells:



from people who took **CDZ173**



with people who took a **placebo**



Answer

Yes, the people who took CDZ173 had less swelling in their lymph nodes and a higher number of certain B cells.

What else was learned?

3 What medical problems happened during this trial?

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

The medical problems that happened in this trial include:



problems in or
around the lungs



common cold or
sinus infection



headache



feeling sick
to the stomach



Answer

Most people had medical problems during both parts of the trial. In Part 2, some were **serious**. Researchers decided CDZ173 was safe for the people in this trial.

Check page 9 of the **main summary** for the 11 **serious** medical problems that happened in Part 2.

Other results

Did the people who got **CDZ173** in this trial have:



... **changes** in how their APDS symptoms affected their health and life?

The doctors' and peoples' answers to questions showed that people had fewer APDS symptoms after taking CDZ173. But, their answers did not show any changes to their life.



... **lower** signs of inflammation?

Some signs of inflammation were lower, but others stayed about the same.



... **less** swelling in other organs, like the spleen?

The people who got CDZ173 in Part 2 had less swelling in other organs, like the spleen. The **spleen** is another organ that helps filter infections out of the blood.



Thank you!

Trials like this cannot happen
without the participants.



Researchers need the results from many trials to find new, possible treatments. This trial is one of many steps on the path to find new and better ways to treat **APDS**.



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

