

# Clinical Trial Summary

## A clinical trial to learn about the safety of the trial drug QBW276 for people with cystic fibrosis

### Thank you!

Novartis, the sponsor of this clinical trial, thanks the participants who helped make this clinical trial possible.

### Trial overview

#### What was the purpose of this trial?



Researchers wanted to learn more about the safety of a trial drug named QBW276 for participants with cystic fibrosis, also called CF. Researchers also wanted to learn how much and how quickly QBW276 got into the blood.

CF is a genetic disease that often leads to life-threatening lung damage and other serious problems.

#### Who was in this clinical trial?



16 adult men and women with CF participated in this clinical trial.

#### What treatments were used in this trial?



In this trial, participants took 2 different amounts of QBW276 or a placebo by using a powder inhaler. A placebo looks like medicine but does not have any medicine in it.

#### What did researchers want to learn?



- What medical problems happened during the trial
- How much and how quickly QBW276 got into participants' blood
- If QBW276 helped participants breathe easier

#### What were the main results of this clinical trial?



8 of the 16 participants had at least 1 medical problem during this trial. None were considered serious and no one stopped the trial early due to a medical problem. Researchers found that QBW276 entered the blood quickly and reached low levels.

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Researchers need many clinical trials to learn if a drug or other treatment is safe and works well. This is a summary of the overall results of this clinical trial. Individual results from each participant may be different and are not included in this summary.

Always talk to a doctor before making any changes to your treatments.

## Why was this clinical trial needed?

Researchers are looking for a better way to treat the breathing problems that happen with cystic fibrosis, also called CF. CF is a genetic disease that causes the body to make thick, sticky mucus that builds up in the lungs, digestive system, and other areas of the body. This buildup can cause life-threatening lung damage and other serious problems. There is currently no cure for CF.

Researchers designed this clinical trial to learn about the safety of the trial drug QBW276 for people with CF. The trial drug QBW276 may help people with CF breathe easier by thinning mucus to prevent buildup in the lungs. QBW276 is breathed in through a powder inhaler.

### **In this clinical trial, the researchers wanted to learn:**

- What medical problems happened during the trial
- How much and how quickly QBW276 got into participants' blood
- If QBW276 helped participants breathe easier

## Who was in this clinical trial?

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16 men and women with CF participated in this clinical trial. They were all between 22 to 49 years old and were 34 years old on average.

Every participant in this clinical trial had CF and was otherwise healthy based on a doctor's exam. No one could be in this clinical trial if they had a lung transplant.

Researchers conducted this clinical trial in the United States and Germany.

This clinical trial began in September 2017. In April 2018, the trial ended early because the sponsor decided to stop all research on QBW276 in people with CF. The decision to stop was not related to the safety of the trial drug.

The participants already enrolled in this clinical trial were allowed to finish. They began on different dates, and all participants completed the trial.



For more information about who could and could not be in this clinical trial, visit [novctrd.com](http://novctrd.com). Use clinical trial number CQBW276X2201 to find the scientific summary.

## What type of clinical trial was this?

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This was a Phase 2 clinical trial which tests a trial drug's safety and how well it works in a small number of participants.

Researchers used a computer program to randomly assign each participant a treatment: QBW276 or a placebo. A **placebo** looks like medicine but does not have any medicine in it. Using a placebo helps researchers better understand the actual effects of a trial drug.

Participants, trial staff, and sponsor staff did not know who got the trial drug or the placebo. Some clinical trials are done this way because knowing what treatment participants get can affect the results. Not knowing what treatment participants get helps make sure the results are looked at fairly.

## What happened during this clinical trial?

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### Before treatment

Trial doctors checked each participant's health to make sure they could be in this clinical trial. They also measured how easily participants could breathe and took blood samples.

## During treatment

Researchers divided participants into Group 1 and Group 2. The researchers started by testing a lower dose of QBW276 in Group 1 for 7 days. After participants in Group 1 took their last treatment, trial doctors checked for any medical problems. After they made sure no serious problems happened, they tested a higher dose of QBW276 with Group 2 for 14 days.

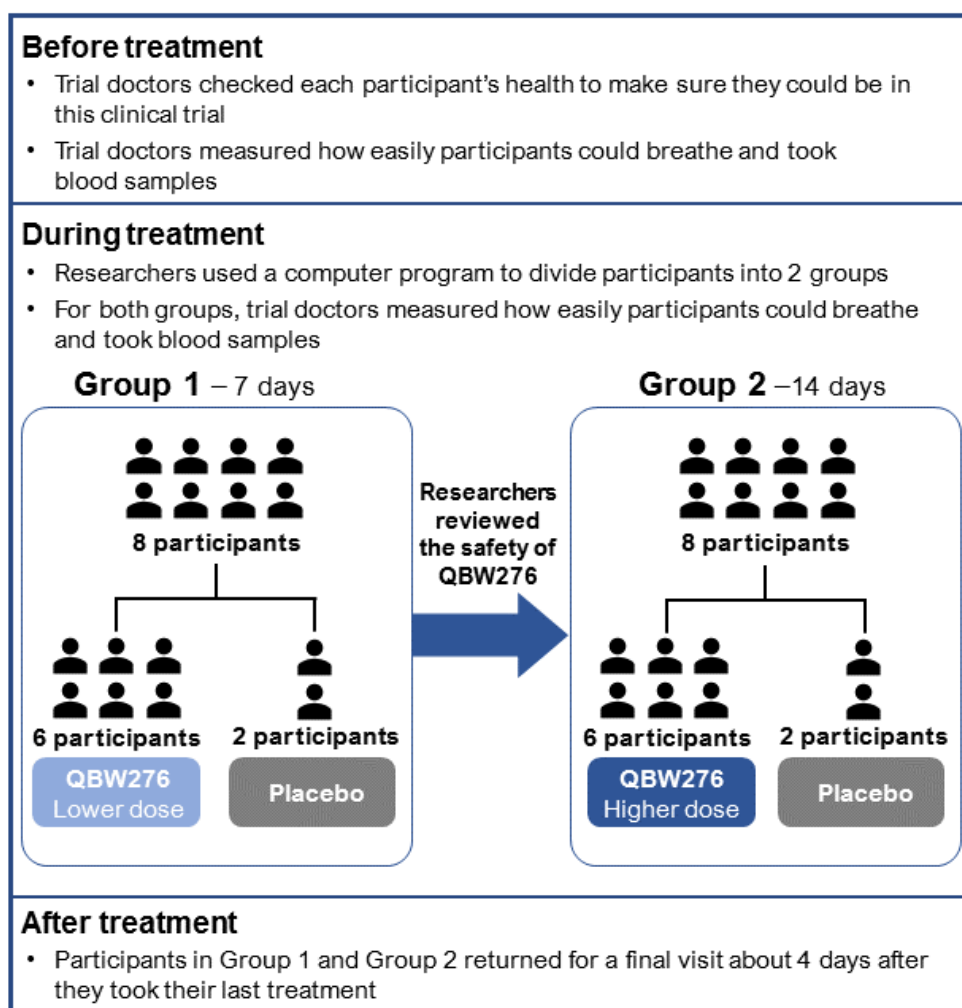
After the first dose of their assigned treatment, trial doctors took blood samples. Participants returned for up to 3 visits while taking their assigned treatment.

## After treatment

Participants in Group 1 and Group 2 returned for a final visit about 4 days after they took their last treatment.

Researchers planned a Group 3 to this trial, but the sponsor decided to stop all research on QBW276 for people with CF.

## How researchers designed this trial:



# What did researchers learn in this clinical trial?

## What medical problems happened during the trial?

Medical problems that happen during clinical trials are called “adverse events”. An **adverse event** is any unwanted sign or symptom that participants have during a trial. An adverse event is considered “serious” when it is life-threatening, causes lasting problems, or the participant needs hospital care.



Adverse events may or may not be caused by treatments in the trial. Many trials are needed to know if a drug or treatment causes an adverse event. Trial doctors keep track of all adverse events that happen in trials, even if they do not think the adverse events might be related to the trial treatments.



Researchers learned 8 of the 16 participants (50%) in this trial had at least 1 adverse event. None of the adverse events were serious. No one stopped taking part in this trial due to an adverse event.

Trial doctors at each location kept track of adverse events. They looked for any adverse events when they checked participants’ blood samples and during the visits. Participants also reported adverse events. The researchers compared the number of participants with adverse events who got QBW276 to those who got the placebo.

No one left this clinical trial or stopped taking their assigned treatment due to an adverse event.

### Participants who had adverse events during this clinical trial

	<b>QBW276 Lower dose</b> (out of 6 participants)	<b>QBW276 Higher dose</b> (out of 6 participants)	<b>Placebo Groups 1 and 2</b> (out of 4 participants)
Participants who had at least 1 adverse event	33% (2)	100% (6)	0% (0)
Participants who had a serious adverse event	0% (0)	0% (0)	0% (0)
Participants who left this trial due to adverse events	0% (0)	0% (0)	0% (0)

## What serious adverse events happened during this trial?



An adverse event is called serious when it is life-threatening, requires a hospital stay, can cause disability or permanent damage, or can cause a birth defect.

During this clinical trial, no one reported serious adverse events and no one died.





## What types of adverse events did participants report?

Some participants reported medical problems or adverse events that were not serious. This section reports the most common adverse events participants had during this clinical trial.

This summary only includes types of adverse events that were reported:

- In 3 or more of the participants in this clinical trial
- From the start of treatment to the end of the clinical trial

### Participants reporting adverse events by type

	<b>QBW276</b> Lower dose out of 6 participants	<b>QBW276</b> Higher dose out of 6 participants	<b>Placebo</b> Groups 1 and 2 out of 4 participants
<b>Cough</b>	0	 4 (67%)	0
<b>Headache</b>	0	 3 (50%)	0
<b>Trouble breathing</b> (dyspnea)	 1 (17%)	 2 (33%)	0



For more information about the adverse events that participants reported in this clinical trial, visit [novctrd.com](https://novctrd.com). Use clinical trial number CQBW276X2201 to find the scientific summary.

## How much QBW276 got into participants' blood?



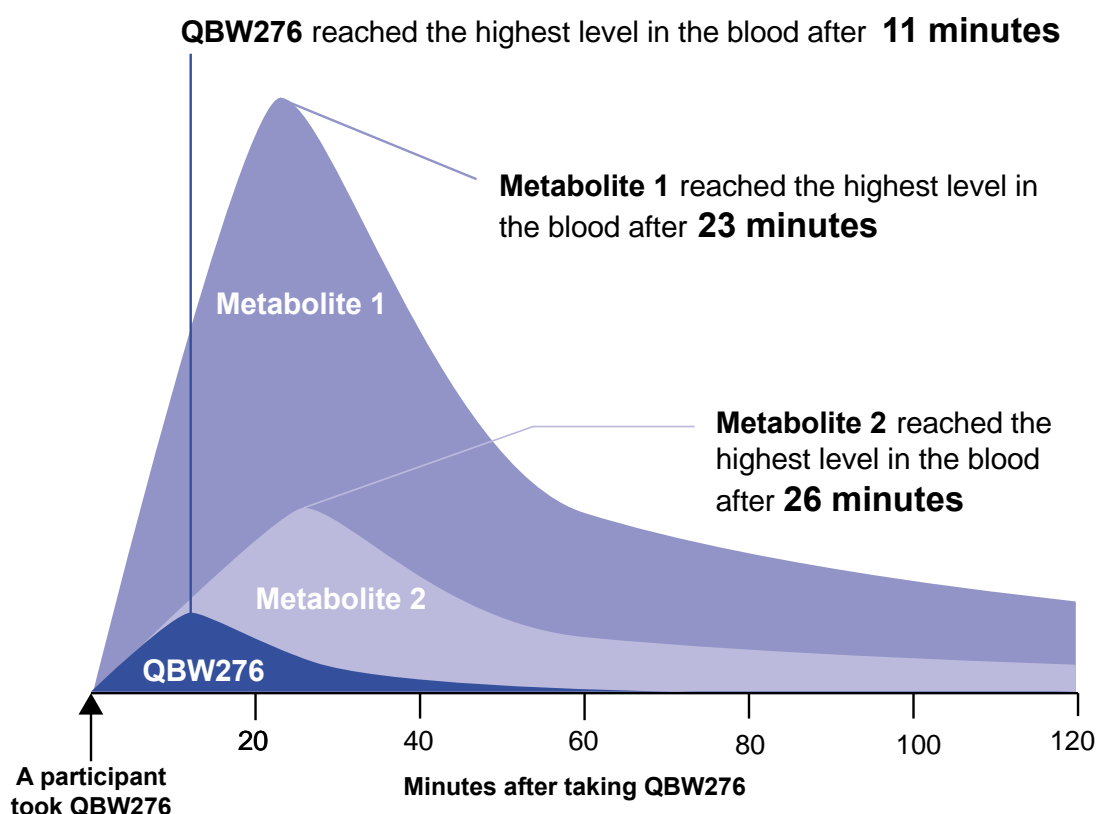
Researchers found QBW276 entered the blood quickly and reached expected levels.

To see how much QBW276 got into participants' blood, researchers took blood samples to measure the levels of QBW276 and its 2 metabolites. Metabolites are substances that form when QBW276 is broken down by the body. This helps researchers decide how much and how often someone may need to take a treatment.

Researchers found:

- Low levels of QBW276 in participants' blood
- QBW276 quickly got into participants' blood after they took it
- The body quickly removed QBW276 and its metabolites from the blood

### The levels of QBW276 and its metabolites in participants' blood over time



## What other key results did researchers learn?



Researchers learned that QBW276 did not have a meaningful effect on how easily participants could breathe compared to the placebo.

To see if QBW276 helped participants breathe easier, researchers used 2 measures:

- **Forced Expiratory Volume during 1 second (FEV<sub>1</sub>):** How many liters of air a person can exhale (blow out) with force in 1 second. A higher FEV<sub>1</sub> means that a person can breathe more easily.
- **Lung Clearance Index (LCI):** How evenly air goes in and out of the lungs. A lower LCI means that a person's lungs work more evenly.

Based on the small number of participants in this clinical trial, researchers can't conclude if there was a meaningful difference in participants' FEV<sub>1</sub> and LCI results from before and after treatment.

## How has this clinical trial helped?

The results of this clinical trial helped researchers learn more about the safety and effects of QBW276. The results also help researchers know how much and how often someone would need to take QBW276 to have an effect.



The results presented here are for a single clinical trial. No single clinical trial can give a complete picture of the benefits and risks of a trial drug. The results of many trials are needed to find out which treatments can be used for people with CF. This summary shows only the main results from this trial. Other clinical trials may provide new information or different results.



# Where can I learn more about this and future clinical trials?

If you were in this clinical trial and have questions about the results, speak with the doctor or staff where you took part in this clinical trial.



This is a summary of the results for one clinical trial.

You can find detailed results and more information about this clinical trial on the Novartis Clinical Trial Results website:

1. Visit [novctrd.com](https://novctrd.com)
2. Click on “Clinical trial results and trial summary for patients” at the top right of the page
3. Read and scroll down, then click “I accept” to agree to use the information and the website
4. Select “Search by study number” on the bottom left of the page
5. Type “**CQBW276X2201**” in the search box and click search

This clinical trial was registered on the following website:

- ClinicalTrials.gov – <https://clinicaltrials.gov/>  
To find this trial, type **CQBW276X2201** in the **Other terms** search box
- European Union Clinical Trials Register – <https://www.clinicaltrialsregister.eu/ctr-search>  
To find this trial, type **CQBW276X2201** in the search box

**Full trial title:**

A randomized, double blind, placebo-controlled study to assess the safety, tolerability, pharmacokinetics, and pharmacodynamics of multiple doses of inhaled QBW276 in patients with cystic fibrosis

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## Thank you!

Novartis would like to thank all of the trial participants that made this clinical trial possible. The trial participants helped researchers answer important health questions and test a possible medical treatment. Many volunteers and many clinical trials are needed to advance medical science.

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