

# A clinical trial to learn about the safety and effect of the trial drug QCC374 on high blood pressure in the lungs



## Thank you!

**Thank you to the participants** who took part in the clinical trial for the drug **QCC374**. All of the participants helped the researchers learn more about how QCC374 works and how safe it is to take.

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. An independent organization prepared this summary of the trial results.

We hope this helps the participants understand their important role in medical research.

## Trial information

**Trial number:** QCCC374X2201

**Drug studied:** QCC374

**Sponsor:** Novartis

You can find **more information** about this trial by going to the websites listed on **page 9** of this summary.



If you participated in the trial and have **questions** about the results, please speak with the trial doctors or staff at your trial site.

## Trial overview

### What was the purpose of this trial?

[Read more on page 3](#)



This clinical trial was designed to learn about the safety of the trial drug QCC374 for people with pulmonary arterial hypertension, also called PAH. The clinical trial team also wanted to learn about the effect of QCC374 on high blood pressure in the lungs of people with PAH.

#### This trial was designed to answer these questions:

- Did QCC374 lower blood pressure in the arteries of the lungs?
- What medical problems did the participants have during the trial? Keeping track of the medical problems helped learn about the safety of QCC374.

### Who was in this trial?

[Read more on page 3](#)



- 8 men and women were in this clinical trial
- All the participants had PAH

### What treatments did the participants take?

[Read more on page 4](#)

The participants were assigned one of these treatments to take with a powder inhaler:

- QCC374
- Placebo – looks like the trial drug but does not have any trial drug in it

### What were main results of this trial?

[Read more on page 5](#)



- The clinical trial team found no new safety concerns during this trial.
- This trial stopped early because the sponsor decided to stop all research on QCC374 for people with PAH. The decision to stop was not related to safety.
- Because the trial ended early, there were too few participants for the clinical trial team to conclude if QCC374 lowered blood pressure in the lungs.

## What was the purpose of this trial?

Researchers are looking for a better way to treat pulmonary arterial hypertension, also called PAH. **PAH** is a rare condition that causes high blood pressure in the lungs. In PAH, high blood pressure happens when small blood vessels called arteries become narrowed. As blood pressure rises, the heart must work harder to pump blood to the lungs.

Symptoms of PAH include:

- Feeling tired
- Trouble breathing
- Dizziness
- Feeling light-headed or fainting

Symptoms of PAH often get worse over time and can be life-threatening. There is no cure for PAH, but some medicines may help treat the symptoms.

The trial drug **QCC374** is designed to be directly breathed into the lungs to open the arteries and lower blood pressure. This can lessen stress on the heart. Before a drug can be approved for patients to take, researchers do many clinical trials to find out how safe it is and how it works.

**This clinical trial was designed to learn:**

- Did QCC374 lower blood pressure in the arteries of the lungs?
- What medical problems did the participants have during the trial?

## Who was in this trial?

8 participants began this clinical trial – 7 women and 1 man. The participants' ages ranged from 20 to 65 years old. They were 45 years old on average.

Every participant in this clinical trial had PAH.

This clinical trial took place in Germany, the Republic of Korea, the United Kingdom, and the United States.



For more information about who could and could not be in this clinical trial, and the participants in this trial, visit [novctrd.com](https://novctrd.com). Use clinical trial number **CQCC374X2201** to find the scientific summary.

# What treatments did the participants take?



The clinical trial team used a computer program to randomly assign each participant one of these treatments:

- QCC374
- Placebo

The team used a computer program to help make sure that comparing the results of the treatments was as fair as possible.

The participants used a powder inhaler 2 times a day to take either QCC374 or the placebo. A **placebo** looks like the trial drug but does not have any trial drug in it. Using a placebo helps to better understand the actual effects of a trial drug.

The participants assigned QCC374 took a low dose to start. For 2 weeks, the trial doctors could increase their dose to a medium dose, and then to a high dose. For the next 14 weeks, the participants continued at the highest dose they could take without major issues. During this time, trial doctors could adjust a participants' dose as needed.

In addition to their assigned treatment, the participants were allowed to keep taking their usual PAH medicine unless it could cause problems with QCC374.

The participants and trial staff did not know what treatment each participant received during the trial. Some clinical trials are done this way because knowing what treatment participants get can influence the results. Not knowing what treatment participants get helps make sure the results are looked at fairly.

## What happened during this clinical trial?

---

This clinical trial began in September 2017. The participants in this clinical trial began on different dates. In June 2018, the clinical trial team stopped this trial early because the sponsor decided to stop all research on QCC374 for people with PAH. The decision to stop was not related to safety. 2 participants did not complete this trial.

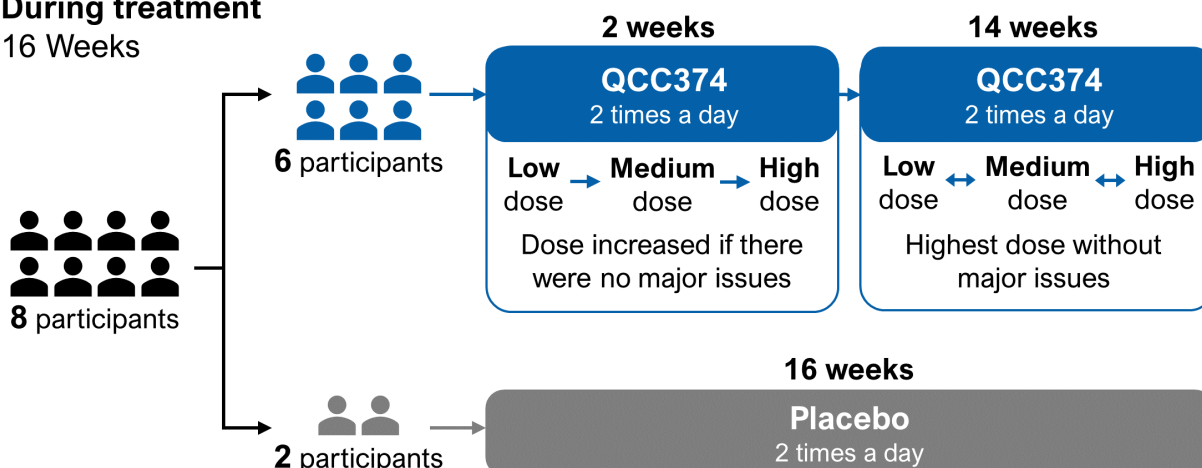
## How this trial was designed:

### Before treatment

- The participants had the blood pressure in their lungs measured
- The trial doctors checked each participant's health to make sure they could be in this clinical trial

### During treatment

16 Weeks



- Trial doctors measured the blood pressure in participants' lungs 2-4 hours after their last dose

### After treatment

- The participants had the option to join another related clinical trial after completing this trial
- The participants who didn't join had a final visit about 4 weeks after their last dose

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



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. Researchers need many clinical trials to learn if a drug or other treatment is safe and works well. This is a summary of only one clinical trial. You should not use the results of this clinical trial to make decisions about your health care. Always talk to a doctor before making any changes to your health care.

## Did QCC374 lower blood pressure in the arteries of the lungs?



Because the trial ended early, there were too few participants for the clinical trial team to conclude if QCC374 lowered blood pressure in the lungs.

The clinical trial team compared the blood pressure in the participants' lungs before and after treatment. They also compared the change in blood pressure for the participants who took QCC374 to the participants who took the placebo. The team was unable to conclude if QCC374 lowered blood pressure in the lungs.

## What medical problems did participants have 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.



The clinical trial team found no new safety concerns during this trial. The most common adverse event was headache.

Trial doctors looked for any adverse events when they checked the participants' blood and urine samples and during the visits to the trial site. The participants also reported adverse events. Then, the researchers compared the number of participants with adverse events who got QCC374 to those who got the placebo.

## Adverse events during this trial

Participants who had:	QCC374 (out of 6 participants)	Placebo (out of 2 participants)
Adverse events	 <b>100% (6)</b>	 <b>50% (1)</b>
Serious adverse events	 <b>17% (1)</b>	 <b>0%</b>
Left this trial due to adverse events	 <b>17% (1)</b>	 <b>0%</b>

### What serious adverse events did the participants have?

One participant had a serious adverse event during this clinical trial. This was:

- **Syncope:** fainting

No other participants reported serious adverse events, and no participants died during this trial.

### What types of adverse events did the participants have?

Some participants reported adverse events that were not serious.













This section reports the types of adverse events that were:

- Most common: adverse events that happened to 2 or more participants
- Less common: adverse events that happened to only 1 participant











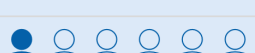







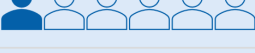







For more information about the adverse events reported by the participants in this clinical trial, visit [novctrd.com](https://novctrd.com). Use clinical trial number **CQCC374X2201** to find the scientific summary.

## Most common types of adverse events

	QCC374 (out of 6 participants)	Placebo (out of 2 participants)
Headache	 83% (5)	 0%
Feeling sick to the stomach Nausea	 50% (3)	 0%
Common cold Nasopharyngitis	 33% (2)	 50% (1)
Redness in the face or other skin areas Flushing	 50% (3)	 0%
Diarrhea	 33% (2)	 0%
Jaw pain	 33% (2)	 0%

## Less common types of adverse events

	QCC374 (out of 6 participants)	Placebo (out of 2 participants)
Cavities	 0%	 50% (1)
Change in sense of taste Dysgeusia	 17% (1)	 0%
Ear infection	 17% (1)	 0%
Gastrointestinal disorders such as constipation or heartburn	 17% (1)	 0%
GERD GastroEsophageal Reflux Disease	 17% (1)	 0%
Hangover	 0%	 50% (1)
Head discomfort	 17% (1)	 0%
Pain in the shoulders, arms, legs, or neck	 17% (1)	 0%
Skin redness Erythema	 17% (1)	 0%
Stomach flu	 17% (1)	 0%
Throwing up Vomiting	 17% (1)	 0%
Upset stomach	 17% (1)	 0%



## What was learned from this trial?

Even when a clinical trial stops early, researchers still learn from the information collected. The clinical trial team learned more about the safety of QCC374 by finding no new safety concerns.



The results presented here are for one clinical trial. One clinical trial cannot 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 PAH. 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?



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 “**CQCC374X2201**” in the search box and click search



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

This clinical trial was registered on the following websites:

- ClinicalTrials.gov – <https://clinicaltrials.gov/>

To find this trial, type **CQCC374X2201** in the **Other terms** search box

### Full trial title:

A randomized, parallel-group, placebo-controlled subject and investigator blinded study to assess the safety, tolerability, pharmacokinetics and efficacy of QCC374 in the treatment of pulmonary arterial hypertension

---

## 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 learn about a possible medical treatment. Many volunteers and many clinical trials are needed to advance medical science.

---



Novartis is a global healthcare company based in Switzerland that provides solutions to address the evolving needs of patients worldwide.

1-888-669-6682 (USA)  
+41-61-324 1111 (EU)  
[www.novartisclinicaltrials.com](http://www.novartisclinicaltrials.com)