Clinical Trial Results Summary

NOVARTIS

A clinical trial extension to learn more about the safety of the trial drug QCC374 for people with high blood pressure in their 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 safe QCC374 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.

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 information

Trial number: CQCC374X2201E1
Drug studied: QCC374
Sponsor: Novartis

You can find **more information** about this trial by going to the website listed on **pages 8-9** of this summary.

Trial overview

What was the purpose of this trial?

Read more on **page 3**

This clinical trial extension was designed to learn more about the safety of the trial drug QCC374 for people with pulmonary arterial hypertension, also called PAH. This trial was also designed to learn more about the effects of QCC374.

This trial was mainly designed to answer this question:

- What medical problems did participants have during the trial? Keeping track of medical problems helped learn about the safety of QCC374.

Who was in this trial?

Read more on **page 3**

- 5 women with PAH began this clinical trial
- All participants took part in an earlier clinical trial on QCC374

What treatment did participants take?

Read more on **page 4**

All participants took QCC374 using a **powder inhaler.**

What were main results of this trial?

Read more on **page 6**

The clinical trial team found no new safety concerns during this trial.

This trial had other results along with the main results**.**

Read more on **page 7**

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 people to take, researchers do many clinical trials to find out how safe it is and how it works.

This trial was designed to answer these questions:

- What medical problems did participants have during the trial?
- How far could participants walk in 6 minutes after treatment with QCC374?
- How well could participants' hearts pump blood to their lungs?
- How much and how fast did QCC374 get into participants' blood?

Who was in this trial?

5 participants began this trial – all were women. Participants' ages ranged from 37 to 65 years old. They were 47 years old on average.

Every participant in this trial had PAH.

This was a trial extension. This means that participants from an earlier trial on QCC374 were invited to this trial to learn more about QCC374.

This trial took place in Germany, the United Kingdom, and the United States.

For more information about who could and could not be in this trial, and the participants in this trial, visit novctrd.com. Use trial number **CQCC374X2201E1** to find the scientific summary.

What treatment did participants take?

All participants took QCC374 using a powder inhaler. The dose that each participant took was different and was based on how their body responded to QCC374.

Participants who took QCC374 in the earlier trial continued to take the same dose of QCC374.

Participants who took a placebo in the earlier trial started by taking a low dose of QCC374 in this trial. A placebo looks like the trial drug, but has no trial drug in it. For 2 weeks, the trial doctors could increase their dose to a medium dose, and then to a high dose. For the rest of their treatment, the participants continued at the highest dose they could take without major issues. Trial doctors could adjust a participants' dose as needed.

All participants and trial staff knew what treatment each participant took.

What happened during this trial?

This trial began in February 2018. The participants in this trial began on different dates.

In November 2018, 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.

The clinical trial team planned for participants to take QCC374 for 720 days or about 2 years. Because the trial ended early, the length of time participants took QCC374 ranged from 14 to 220 days.

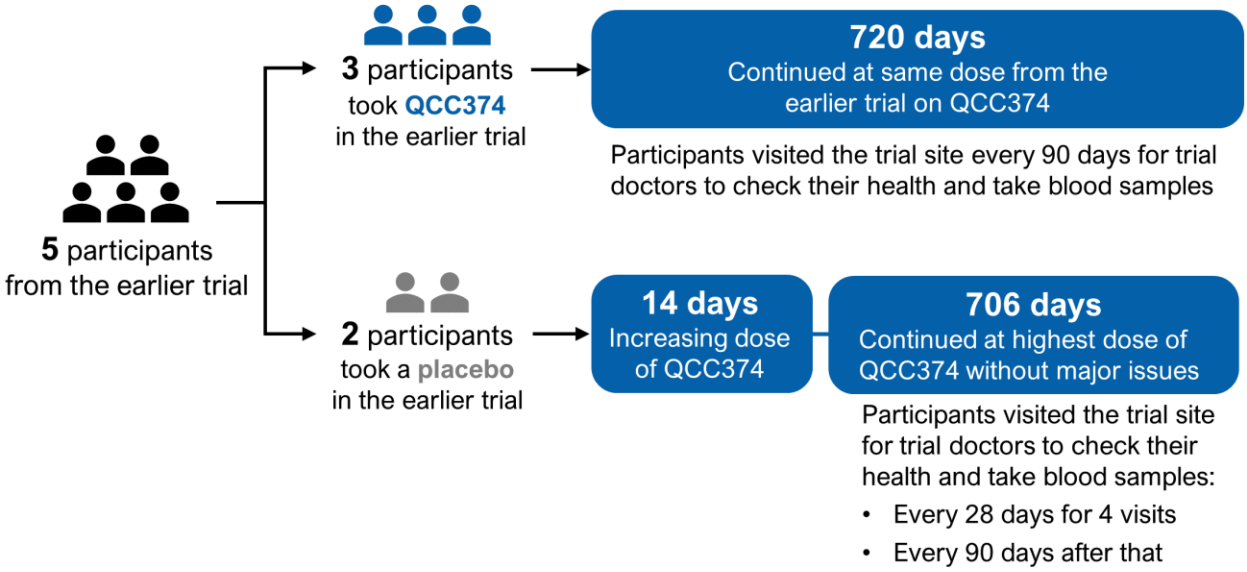
How this trial was designed:

Before treatment

- All the participants took part in an earlier trial on QCC374
- Trial doctors collected a participant's health information from the earlier trial, including how far they could walk in 6 minutes and how well their heart could pump blood to their lungs

During treatment

Planned to last 720 days or about 2 years



After treatment

- Participants had a final trial site visit to check their health 30 days after their last dose
- During this visit, trial doctors also measured how far a participant could walk in 6 minutes and how well their heart could pump blood to their lungs

What were the main results of this trial?

This is a summary of the overall results of this 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.

What medical problems did participants have during the trial?

Medical problems that happen during clinical trials are called **adverse events**. Trial doctors looked for any adverse events when they checked participants' health during the trial. Participants also reported adverse events.

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.

What is an adverse event?

- An **adverse event** is any unwanted sign or symptom that participants have during a trial
- It 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

The clinical trial team found no new safety concerns during this trial. The most common adverse events were headache and common cold. One participant had a serious adverse event, which was PAH that got worse.

Trial doctors looked for any adverse events when they checked participants' health during the visits to the trial site. Participants also reported adverse events.

Adverse events during this trial

Participants who had:	QCC374 (out of 5 participants)
Adverse events	**60%** (3)
Serious adverse events	**20%** (1)
Left this trial due to a serious adverse event	**20%** (1)

What serious adverse events did participants have during this trial?

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

- PAH that got worse

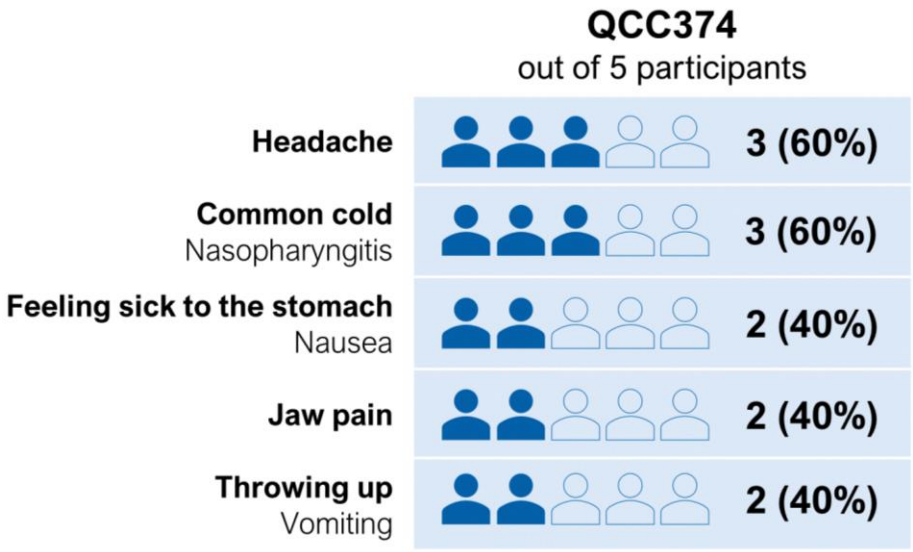
The participant left the trial because of this serious adverse event.

No other serious adverse events were reported and no one died during this trial.

What other adverse events did participants have during this trial?

Some participants reported adverse events that were not serious. This section reports the types of adverse events that happened to **2 or more participants**. Other adverse events happened, but these were reported by only one participant.

Most common adverse events, by type



For more information about the adverse events reported by participants in this trial, visit novctrd.com. Use trial number **CQCC374X2201E1** to find the scientific summary.

What other results were learned?

Because the trial ended early, the number of participants was too small for the clinical trial team to conclude if QCC374 had a meaningful effect on:

- How far participants could walk in 6 minutes
- How well participants' hearts could pump blood to their lungs

The team was also not able to conclude how much and how fast QCC374 got into participants' blood.

What was learned from this trial?

This trial helped learn more about the safety of QCC374. The clinical trial team found no new safety concerns during this trial.

Because the trial ended early, there were too few participants to reach a conclusion about the effects of QCC374 on PAH. The decision to stop was not related to safety.

The results presented here are for one clinical trial. One 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 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 trial.

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

1. Visit 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 “**CQCC374X2201E1”** in the search box and click search

If you would like to view the website in a language other than English, you can click the “Google Translate” button on the top right of the page.

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

This trial was registered on the following websites:

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

  To find this trial, type **CQCC374X2201E1** in the **Other terms** search box

Full trial title:

Long-term, open label, multicenter, extension study to evaluate the safety and tolerability of QCC374 in patients with pulmonary arterial hypertension (PAH)

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.

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

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