Clinical Trial Results

NOVARTIS

Research Sponsor: Novartis

Treatment Studied: Indacaterol

Protocol Number: CQVM149B2203

Thank you!

Thank you for taking part in the clinical trial for the treatment indacaterol. You and all of the participants helped researchers learn more about how indacaterol works in people with asthma.

Novartis sponsored this trial and believes it is important to share the results of the trial with you and the public. An independent non-profit organization called CISCRP prepared this summary of the trial results for you. We hope it helps you understand your important role in medical research.

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

What has happened since the trial ended?

You were in the trial for about 3 months. But, the whole trial took about 4 months to finish. This is because the participants started and stopped the trial at different times. The trial started in September 2017 and ended in January 2018.

The trial included 54 participants in the United States. After the trial ended, the sponsor reviewed the data and created a report of the results. This is a summary of that report.

Why was the research needed?

Researchers are looking for a better way to treat asthma. People with asthma have swelling in the airways of their lungs. This swelling causes the airways to tighten, which decreases the airflow in and out of the lungs. This makes it harder to breathe and can also cause coughing, wheezing, and shortness of breath.

Indacaterol is a drug that helps participants breathe more easily by relaxing and opening up the airways in the lungs. In this trial, the researchers wanted to study 2 forms of the trial drug:

- **Indacaterol maleate** is available as a treatment in many countries around the world, including the United States, for patients with chronic obstructive pulmonary disease, also called COPD.
- **Indacaterol acetate** is the form of indacaterol used in a new trial drug called QVM149. Indacaterol acetate is a new asthma treatment not yet approved for doctors to prescribe to patients.

In this trial, the researchers wanted to know if both indacaterol maleate and indacaterol acetate help participants breathe more easily. The researchers also wanted to know if similar amounts of each form of the drug enter the participants' blood.

The main questions the researchers wanted to answer in this trial were:

- Did the 2 forms of indacaterol change how easily the participants could breathe?
- What other measurements did the researchers use to learn how the participants could breathe?
- How much of the 2 forms of indacaterol got into the participants' blood?
- What medical problems did the participants have?

To answer the questions in this trial, the researchers asked for the help of participants who had asthma for at least 1 year before joining this trial. The men and women in the trial were 26 to 70 years old when they joined.

What kind of trial was this?

This was a “double-blind” trial. This means none of the participants, trial doctors, trial staff, or sponsor staff knew what treatment each participant took. Some trials are done this way because knowing what treatment the participants are taking can affect the results of the trial.

In this trial, the researchers compared indacaterol maleate and indacaterol acetate to a placebo. A placebo looks like a trial drug but does not have any medicine in it. Using a placebo helps researchers better understand the actual effect of a trial.

The researchers used a computer program to randomly choose the order that each participant took the treatments. This helped make sure that comparing the results of the treatments was as fair as possible.

When the trial ended, the sponsor found out which treatment each participant took so they could create a report of the results. The sponsor staff did not know the identity of any of the participants.

What happened during the trial?

Before treatment started, the trial doctors did tests to make sure the participants could take part in the trial. This part lasted up to 14 days. The doctors:

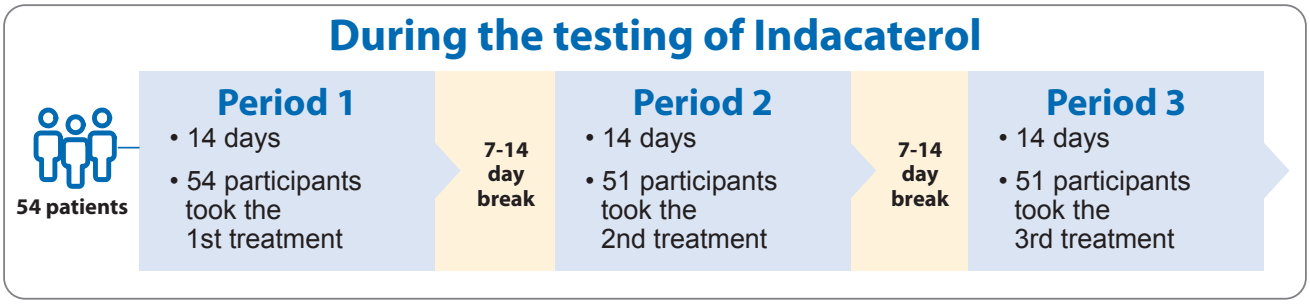
- checked the participants’ overall health and asked what medicines the participants were taking
- taught the participants how to use the treatment inhalers
- taught the participants how to blow into a peak expiratory flow meter, also known as a PEF meter

During treatment, the participants took the trial treatments through an inhaler once a day for 14 days. Treatment was done in 3 periods. In 1 period, each participant took 150 micrograms, also called µg, of one of the following treatments:

- indacaterol maleate
- indacaterol acetate
- placebo

The participants waited 7 to 14 days after taking 1 treatment before taking the next treatment. By the end of the trial, each participant had taken all 3 trial treatments but in different orders.

A total of 54 participants took treatment during Period 1, but then 3 participants left the trial before starting Period 2.



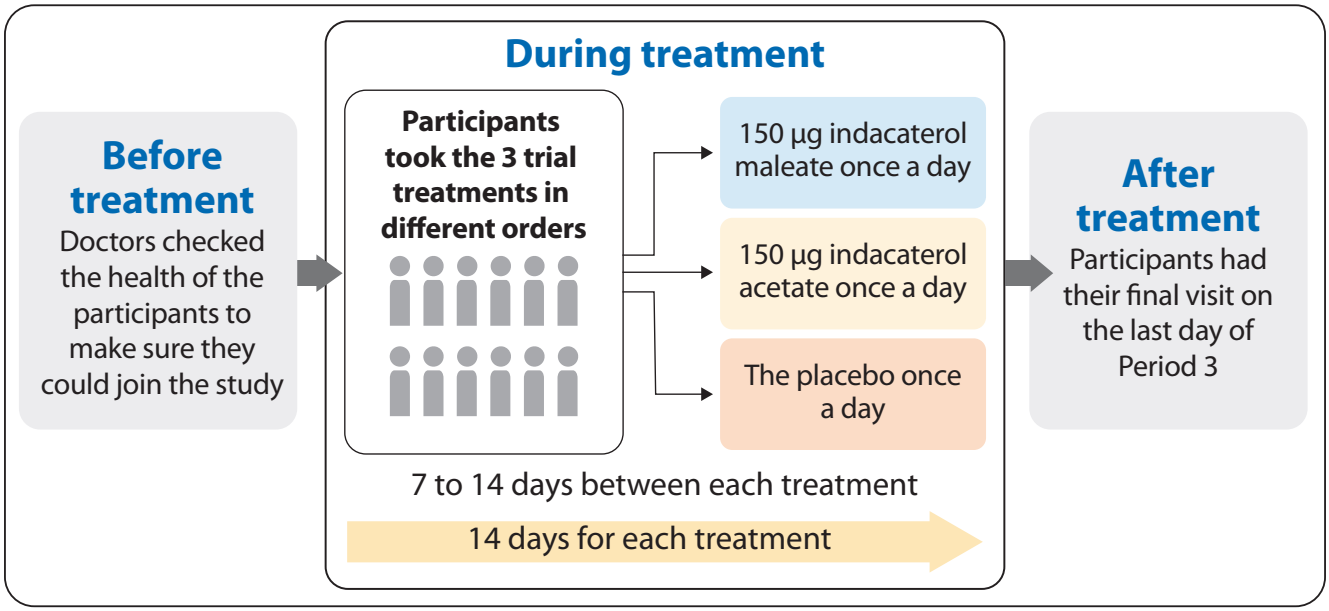
For each trial treatment, the participants visited the trial site to take the first dose. For the next 12 days, the participants took the trial treatment at home. The participants came back to the trial site for their final dose of each trial treatment, and they stayed overnight.

Throughout the trial, the participants took their regular steroid inhaler prescribed by their doctor. The participants used their electronic diaries to record how often they took the trial treatment, their regular steroid inhalers, and any rescue medicine. Rescue medicine is medicine that participants could take if they needed to treat their asthma symptoms right away. In this trial, it was taken through an inhaler.

During the trial, the doctors:

- checked the participants' overall health and asked what medicines the participants were taking
- checked how well the participants could breathe
- took blood and urine samples from the participants
- checked the participants' electronic diaries

The chart below shows how the trial was done.



What were the results of the trial?

This is a summary of the overall results of your trial, not your individual results. The results presented here are for a single trial. Other trials may provide new information or different results. You should not make medical decisions based on the results of a single trial. Always talk to a doctor before making any changes to your medications or treatment plans.

Three participants left the trial before getting all 3 trial treatments. So, the results below may include fewer than 54 participants.

Did the 2 forms of indacaterol change how easily the participants could breathe?

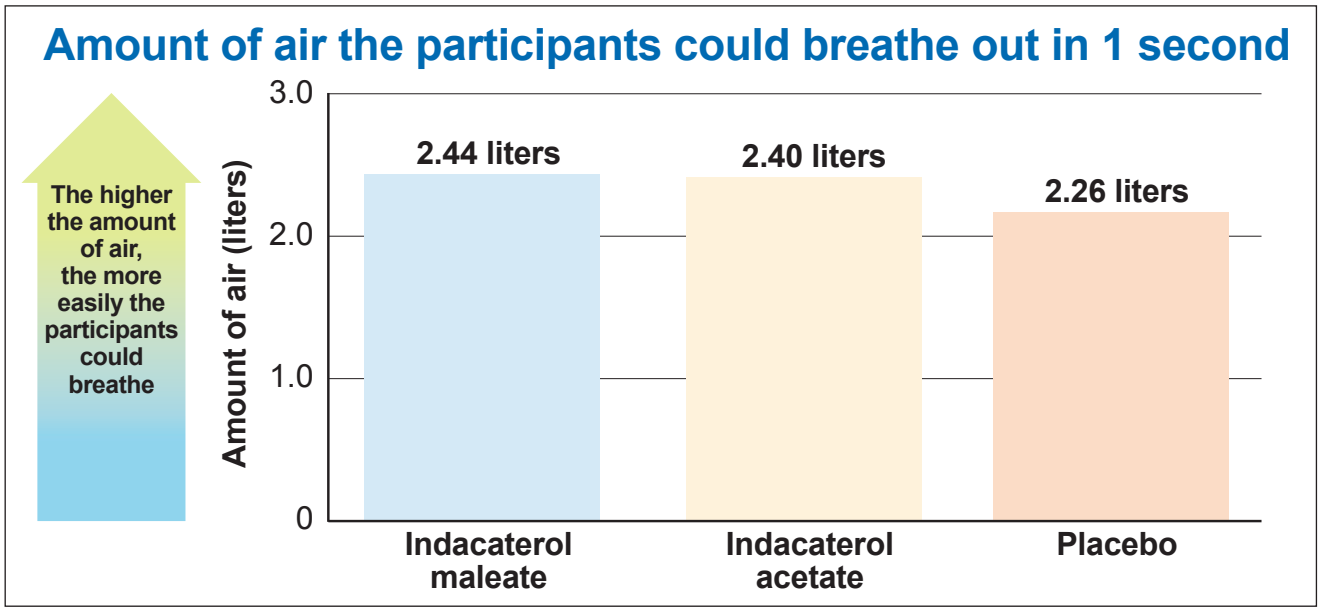
Yes. Overall, the participants could breathe more easily when they took indacaterol maleate or indacaterol acetate compared to when they took the placebo.

Researchers wanted to find out if the effects of indacaterol would last a full 24 hours. To find this out, the researchers measured the amount of air the participants could breathe out in 1 second about 24 hours after taking their last dose of each trial treatment. This amount of air was measured in liters. An increase in this amount meant that the participants could breathe more easily by the end of a 24-hour dosing period.

After 14 days of trial treatment, the researchers found that the average amount of air the participants could breathe out in 1 second 24 hours after their last dose was:

- 2.44 liters after the participants took indacaterol maleate
- 2.40 liters after the participants took indacaterol acetate
- 2.26 liters after the participants took the placebo

The graph below shows the average amount of air the participants could breathe out after 14 days of treatment.



What other measurements did the researchers use to learn how the participants could breathe?

To answer this question, the trial doctors measured the following after 14 days of trial treatment:

- how much air the participants could breathe out in 1 second
- the average amount of air the participants could breathe out in 1 second at different times over 4 hours
- how long it took for the participants to reach the highest amount of air they could breathe out in 1 second
- the total amount of air the participants could breathe out
- the amount of air the participants breathed out during the middle of a forceful exhale
- how quickly the participants could breathe out a full breath of air with force
- how often the participants used any rescue medicines

Overall, the results of these measurements showed that the participants could breathe more easily when they took indacaterol maleate or indacaterol acetate compared to when they took the placebo.

How much of the 2 forms of indacaterol got into the participants' blood?

The researchers wanted to know how much of the indacaterol maleate and the indacaterol acetate got into the participants' blood. To find this out, the researchers measured:

- the average amount of the 2 forms of indacaterol in the participants' blood
- the average highest level of the 2 forms of indacaterol in the participants' blood
- the average amount of time it took to reach the highest level of the 2 forms of indacaterol in the participants' blood

Overall, the researchers found that the average amount and average highest level of indacaterol maleate and indacaterol acetate in the participants' blood were about the same. They also found that the average amount of time it took to reach the highest level in the participants' blood was about the same. This showed the researchers that both forms of indacaterol reached about the same levels in the participants' blood.

What medical problems did the participants have?

Medical problems that happen in 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.

These problems may or may not be caused by the trial treatment. A lot of research is needed to know whether a treatment causes a medical problem. During a trial, all medical problems are reported and written down, whether or not they are caused by the trial treatment. So, when new treatments are being studied, researchers keep track of all medical problems that participants have.

This section is a summary of the adverse events that happened during this trial.

How many participants had adverse events?

In this trial:

- 44.4% of the participants had adverse events. This was 24 of the 54 participants.
- 1.9% of the participants had serious adverse events. This was 1 of the 54 participants.
- 1.9% of the participants left the trial because of an adverse event. This was 1 of the 54 participants.

The table below shows how many participants had adverse events in this trial.

Adverse events in this trial

	Indacaterol maleate (Out of 51 participants)	Indacaterol acetate (Out of 52 participants)	Placebo (Out of 53 participants)	Total (Out of 54 participants)
How many participants had adverse events?	27.5% (14)	13.5% (7)	17.0% (9)	44.4% (24)
How many participants had serious adverse events?	0.0% (0)	1.9% (1)	0.0% (0)	1.9% (1)
How many participants left the trial because of adverse events?	0.0% (0)	1.9% (1)	0.0% (0)	1.9% (1)

What were the most common serious adverse events?

There were 2 serious adverse events that happened during this trial:

- Sinus infection
- Strep throat

No other serious adverse events were reported during this trial. None of the participants died during this trial.

What were the most common adverse events?

In this trial, the most common adverse event was a cough. This happened in 24.1% of the participants. This was 13 of the 54 participants.

The table below shows the adverse events that happened in at least 2 participants in this trial. There were other adverse events, but these happened in fewer participants.

Most common adverse events in this trial

	Indacaterol maleate (Out of 51 participants)	Indacaterol acetate (Out of 52 participants)	Placebo (Out of 53 participants)	Total (Out of 54 participants)
Cough	23.5% (12)	0.0% (0)	1.9% (1)	24.1% (13)
Infection of the upper airways	2.0% (1)	3.8% (2)	3.8% (2)	9.3% (5)
Joint pain	0.0% (0)	1.9% (1)	1.9% (1)	3.7% (2)
Sinus infection	0.0% (0)	1.9% (1)	1.9% (1)	3.7% (2)
Vomiting	2.0% (1)	1.9% (1)	0.0% (0)	3.7% (2)

How has this trial helped participants and researchers?

The information described above helped the researchers better understand if 2 forms of indacaterol worked similarly when compared to the placebo in people with asthma.

The results presented here are for a single trial. This summary shows only the main results from this one trial in a small number of participants. Other trials may provide new information or different results. It takes volunteers in many trials all around the world to advance medical science.

Where can I learn more about this trial?

More information about the results of this trial can be found in the scientific results summary available on the Novartis Clinical Trial Results website (www.novctrd.com). Once on the site, click **"READ MORE"** under **"Clinical trial results"** at the bottom of the page. After agreeing to enter the Novartis website, type **"CQVM149B2203"** into the keyword search box and click **"Search"**. If you have questions about the results, please speak with the trial doctor or staff at your trial site.

You can find more information about this trial on the website listed below:

- www.clinicaltrials.gov. Once you are on the website, type **"NCT03257995"** into the search box and click **"Search"**.

If more clinical trials are planned, they will be listed on the above public website or www.novartisclinicaltrials.com. Search for **"QVM149"**.

Full trial title: A randomized, double-blind, placebo-controlled, three-period cross-over study to assess the pharmacodynamics, safety, tolerability, and pharmacokinetics of two orally inhaled indacaterol salts (maleate and acetate) delivered via the Concept1 inhalation device in participants with asthma

Thank you!

Clinical trial patients belong to a large community of patients around the world. They help researchers answer important health questions and test new medical treatments.

CISCRP

The Center for Information & Study on Clinical Research Participation (CISCRP) is a non-profit organization focused on educating and informing the public about clinical research participation.

CISCRP
One Liberty Square, Suite 1100
Boston, MA 02109

1-877-MED-HERO • www.ciscrp.org

NOVARTIS

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

1-888-669-6682 (US);

+41613241111 (EU)

novartisclinicaltrials.com