A 4-week study to test different doses of BI 1265162 in adolescents and adults with cystic fibrosis using the Respimat® inhaler – BALANCE – CF™1

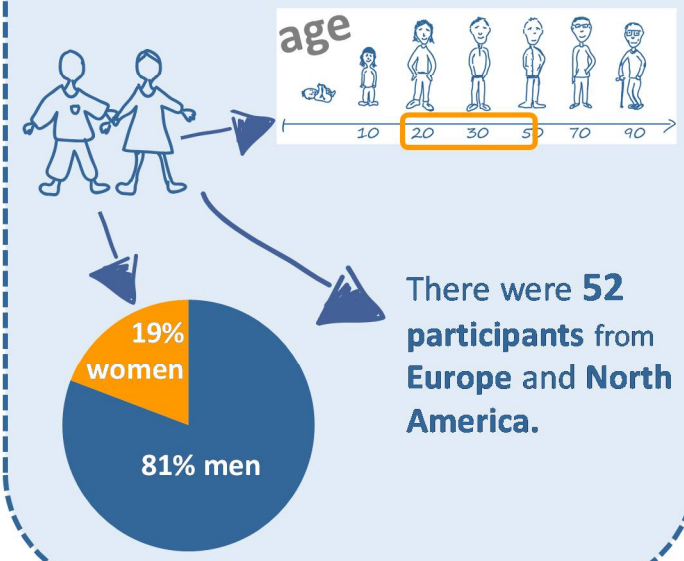
Boehringer Ingelheim

Cystic fibrosis (CF) is a disease of the **glands** that produce mucus and sweat. CF makes the mucus in the lungs **thicker**, and this makes **breathing difficult**.

This **study** was to find out:

Does a medicine called **BI 1265162** help people with **cystic fibrosis** by making it easier for them to breathe?

Participants had cystic fibrosis



There were **52 participants** from **Europe** and **North America.**

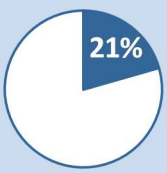
Participants were divided into **5** groups.

They inhaled twice a day for 4 weeks:

BI 1265162 in one of the following doses:

20 µg / 50 µg / 100 µg / 200 µg
or
Placebo,
which looked like BI 1265162 but didn't contain any medicine.

21% of participants who took BI 1265162 and **17%** of participants who took placebo had **unwanted effects.**



7 out of 34 participants

3 out of 18 participants

RESULTS

The **study** was **stopped early** after a planned analysis from the first 28 participants.

Results from this analysis showed that the chance of BI 1265162 working better than placebo **was too small to continue**.

Study: 1399-0003

A 4-week study to test different doses of BI 1265162 in adolescents and adults with cystic fibrosis using the Respimat® inhaler – BALANCE – CF™1

This is a summary of results from 1 clinical study.

We thank all study participants. You helped us to answer important questions about BI 1265162 and the treatment of cystic fibrosis.

What was this study about?

The purpose of this study was to find out whether a medicine called BI 1265162 helps people with cystic fibrosis (CF) by making it easier for them to breathe.

Cystic fibrosis is a disease of the glands that produce mucus and sweat (secretory glands). It is passed from parents to children through genes. The disease affects the lungs and other organs. People with CF have mucus that is thick and sticky. It builds up in the lungs and blocks the airways. This makes it difficult to breathe. People with CF often need treatments to loosen mucus from their airways.

While the study was ongoing, we did a planned analysis of the results. The analysis showed that the chance of BI 1265162 working as we had hoped was too small to continue. For this reason, the study was stopped early.

Who took part in this study?

People 12 years of age and older with CF were to take part in this study. People older than 18 years entered the study first. The study was stopped early, before anyone younger than 18 years old could join.

52 participants took part in the study. 42 were men (81%) and 10 were women (19%). The average age was 31 years old. The youngest participant was 18 years old and the oldest participant was 50 years old. Throughout the study, participants continued to take their usual medications for CF.

The following table shows the numbers of participants in the study in different regions.

Region	Countries	Number of Participants
Europe	Belgium, France, Germany, Spain, Sweden, United Kingdom	41
North America	Canada, United States	11

How was this study done?

The first 28 participants to enter the study were adults. They were divided into 2 groups of equal size:

- Group 1: 200 µg of BI 1265162 breathed in twice a day for 4 weeks
- Group 2: Placebo breathed in twice a day for 4 weeks.

Every participant had an equal chance of being in either group. The participants and doctors did not know whether the participants were in the BI 1265162 group or in the placebo group.

Participants used the Respimat® inhaler to breathe in the medicines. The Respimat® is an inhaler that converts medicine into a soft mist. The placebo inhaler looked like the BI 1265162 inhaler but did not contain any medicine. We compared BI 1265162 with placebo to find out how well BI 1265162 works.

After the first 28 participants entered the study, we did the first planned analysis of the results. We continued to test additional doses of BI 1265162 while the analysis was ongoing. The next participants were divided into 5 groups. All groups used the Respimat® device to inhale the medicine twice a day for 4 weeks. The groups were:

- Group 1: 20 µg of BI 1265162
- Group 2: 50 µg of BI 1265162
- Group 3: 100 µg of BI 1265162
- Group 4: 200 µg of BI 1265162
- Group 5: placebo group

Every participant had an equal chance of being in each group. The participants and doctors did not know whether the participants were in the BI 1265162 groups or in the placebo group.

To compare BI 1265162 with placebo, we used a lung function test called expiratory volume in 1 second (FEV_1). FEV_1 measures the amount of air that a person can forcefully blow out in 1 second after taking a deep breath.

During the study, the participants visited the doctors regularly. At these visits, the doctors collected information about the participants' health.

What were the results of this study?

We checked whether the participants' lung function changed after taking BI 1265162 or placebo for 4 weeks. The results from the first 28 participants showed that the chance of BI 1265162 working better than placebo was too small to continue. So, after review of the results, the study was stopped.

The results from all participants and additional BI 1265162 doses also did not show a difference between BI 1265162 and placebo. But, these results were difficult to interpret. This was because there were not enough participants in each group, due to the trial being stopped earlier than scheduled.

Did participants have any unwanted effects?

Yes, participants in all groups had unwanted effects. Unwanted effects are health problems that the doctors think were caused by BI 1265162 or placebo. In this study:

- 7 out of 34 participants (21%) who received BI 1265162 had unwanted effects
- 3 out of 18 participants (17%) who received placebo had unwanted effects.

None of the unwanted effects were reported for more than 1 participant.

Some unwanted effects were serious because they required a stay in hospital, were life-threatening or fatal. Unwanted effects were also serious if the doctor thought they were serious for any other reason. In this study, 1 participant in the BI 1265162 200 µg group had a serious unwanted effect. 1 participant in the placebo group died from an unwanted effect.

Where can I find more information about this study?

You can find further information about this study at these websites:

1. Go to http://www.mystudywindow.com and search for the study number 1399-0003.
2. Go to www.clinicaltrialsregister.eu/ctr-search and search for the EudraCT number 2019-000261-21.
3. Go to www.clinicaltrials.gov and search for the NCT number NCT04059094.

Boehringer Ingelheim sponsored this study.

The full title of the study is: 'A randomised, double-blind, placebo-controlled and parallel group trial to evaluate efficacy and safety of twice daily inhaled doses of BI 1265162 delivered by Respimat® inhaler as add-on therapy to standard of care over 4 weeks in patients with cystic fibrosis – BALANCE – CF™ 1'.

This was a Phase 2 study. This study started in September 2019 and finished in April 2020.

Are there additional studies?

No additional studies with BI 1265162 are planned. If we do more clinical studies with BI 1265162, you will find them on the websites listed above. To search for these studies, use the term BI 1265162.

Important notice

This lay summary is provided as part of Boehringer Ingelheim's commitment to publicly share clinical study results.

This summary shows only the results from one study and may not represent all of the knowledge about the medicine studied. Other studies may have different results. Usually, more than one study is carried out to find out how well a medicine works and to determine the side effects of a medicine.

This lay summary may include uses, formulations, or treatment regimens for the medicine studied that may be approved or not approved in your country. This lay summary is not intended to promote any product or indication, to guide treatment decisions, or to replace the advice of a healthcare professional.

You should not change your therapy based on the results of this study. Always consult with your treating physician about your therapy.