Clinical Trial Results

AstraZeneca

Research Sponsor: AstraZeneca

Drugs Studied: AZD5634

National Clinical Trial #: NCT02679729

Protocol #: D6600C00001

Study Date: February 2016 to October 2016

Short Study Title: A study in healthy participants to see if AZD5634 is safe to take and how AZD5634 acts in the body

Thank you!

As a clinical study participant, you belong to a large community of participants around the world. You help researchers answer important health questions and discover new medical treatments.

Thank you for taking part in the clinical study for a drug called AZD5634. This drug is being developed to treat cystic fibrosis. You and all of the other participants helped researchers learn if AZD5634 is safe to take and how it acts in the body.

AstraZeneca, the sponsor of this study, thanks you for your help and thinks it is important for you to know the results of your study. An independent non-profit organization called CISCRP prepared this summary of the study results for you with the help of a medical writing organization. We hope it helps you understand and feel proud of your important role in medical research. If you have questions about the results, please speak with the doctor or staff at your study site.

What has happened since my study ended?

Your study started in February 2016 and ended in October 2016. It included 63 participants at 2 study sites in the United States. When the study 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?

Before patients can take a new drug, the company developing it must do research studies to show that the drug is safe and effective. The first step in studying a new drug is to test it in healthy people. This means people without any serious health problems.

The study drug, AZD5634, is being developed to treat cystic fibrosis. Cystic fibrosis affects the cells that make mucus, sweat, and digestive juices. These fluids in the body are normally thin and slippery. But the fluids in people with cystic fibrosis are thick and sticky. This can cause severe damage to the lungs and the digestive system.

In this study, researchers compared AZD5634 with a placebo. A placebo looks like the study drug but contains no real medicine. Researchers use a placebo in studies so that they can compare the results of participants who take study drugs with the results of participants who take no medicine at all.

In this study, researchers wanted to know:

- What medical problems did participants have after they got AZD5634 or the placebo?
- How did AZD5634 act in the body?

What kind of study was this?

Your study was a "single-blind" study. The participants did not know which drug they got, but study staff found out which drug each participant got after the participants' last visit. You and other participants got the study drug or the placebo. Which treatment participants got was decided by chance, like rolling dice. Your study included healthy men and women who were 20 to 50 years old.

What happened during the study?

This study had 2 parts: Part A and Part B. If you were in one part of the study, you could not be in the other part of the study.

During Part A, participants were in the study for up to 3 months. All 57 participants visited their study site 4 times. At the first visit, researchers made sure that participants could join the study. During the second visit, participants stayed at the study site for 4 days. During this time, participants got the study drug or the placebo through a nebulizer. A nebulizer is a machine that turns a liquid medicine into a mist that you can breathe in through a mouthpiece.

The different doses of the study drug were given in micrograms, or µg. This is a widely accepted scientific unit of measurement.

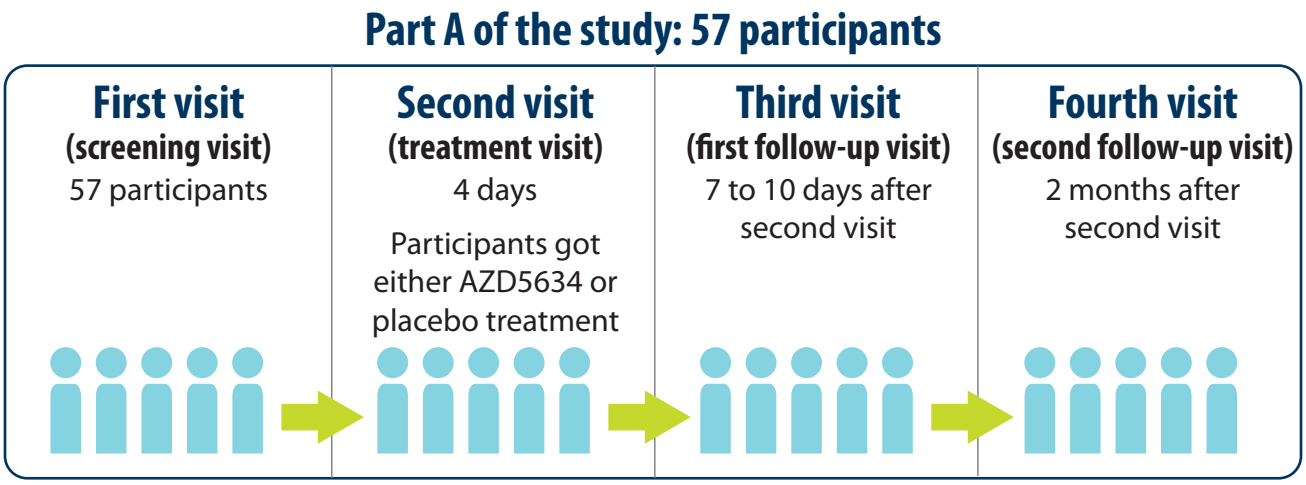
For every 3 participants who got AZD5634, 1 participant got the placebo.

Each participant got 1 of 7 AZD5634 treatments or the placebo:

- 10 µg
- 27 µg
- 81 µg
- 216 µg
- 648 µg
- 1296 µg
- 1692 µg
- placebo

Overall, there were 43 participants who got AZD5634 and 14 participants who got the placebo during Part A.

The figure below shows how Part A of the study was done.



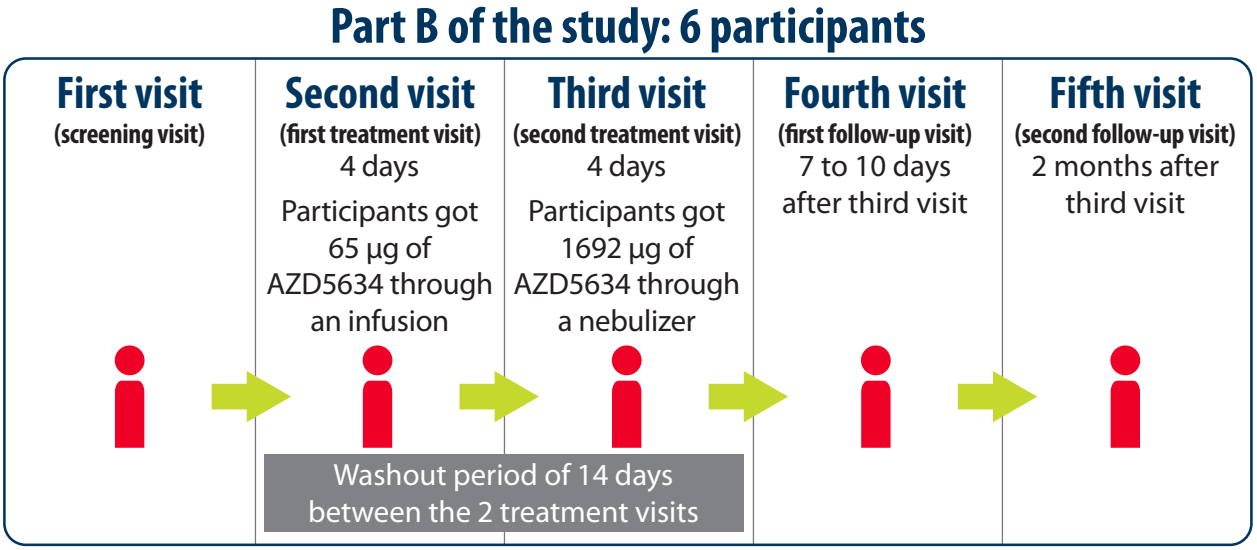
Part B started after Part A ended.

During Part B, participants were in the study for up to 4 months. All 6 participants visited the study site 5 times. At the first visit, researchers made sure participants could join the study. During the second and third visits, participants stayed at the study site for 4 days.

During the second visit, all 6 participants got 65 µg of AZD5634 through a plastic tube put into their vein. This process is called an infusion. During the third visit, all participants got 1692 µg of AZD5634 through a nebulizer.

There was a "washout period" of 14 days between getting the infusion and using the nebulizer. This means that when participants get the next study treatment, the previous drug treatment has been processed and "washed out" from the body. During this time, there are also certain drugs that participants are not allowed to take.

The figure below shows how Part B of the study was done.



During the study, doctors checked the blood pressure, heart rate, temperature, and breathing rate of each participant. Study doctors checked the levels of oxygen in the blood of each participant. Study doctors also tested the blood and urine to make sure that participants were still healthy and to see how AZD5634 was acting in the blood. Study doctors also asked participants how they were feeling. Finally, study doctors checked both the heart and lung health of each participant.

For both Part A and Part B, participants had a follow-up visit 7 to 10 days after their last treatment, and then a final follow-up visit 2 months after their last treatment visit.

What were the study results?

Below is a summary of the results of some of the questions the researchers asked during the study. It is important to know that researchers look at the results of many studies to decide which medicines work best and are safest for patients. Further clinical studies with AZD5634 are currently planned.

What medical problems did participants have during the study?

A lot of research is needed to know whether a drug causes a medical problem. So, researchers keep track of all medical problems that participants have during the study. These medical problems are called "adverse events". They may or may not be caused by the study drug.

How many participants had medical problems during the study?

The tables below and on the next page show how many participants in both Part A and Part B had medical problems. No participants stopped taking the study drug because of a medical problem.

Medical problems in Part A of the study

	AZD5634 10 µg (6 participants)	AZD5634 27 µg (7 participants)	AZD5634 81 µg (6 participants)	AZD5634 216 µg (6 participants)	AZD5634 648 µg (6 participants)	AZD5634 1,296 µg (6 participants)	AZD5634 1,692 µg (6 participants)	Placebo (14 participants)
How many patients developed medical problems?	1 (16.7%)	0	0	1 (16.7%)	1 (16.7%)	0	2 (33.3%)	3 (21.4%)

Medical problems in Part B of the study

	AZD5634 65 µg (Infused) (6 participants)	AZD5634 1,692 µg (Inhaled) (6 participants)
How many patients developed medical problems?	2 (33.3%)	1 (16.7%)

How many participants had serious medical problems?

A medical problem is considered serious when it is life-threatening, causes lasting problems, or needs hospital care. In this study, no participants developed serious medical problems, and no participants died. No new safety concerns were raised during the study.

What were the most common non-serious medical problems in the study?

The tables below show the most common medical problems that happened in both Part A and Part B of the study.

Most common non-serious medical problems in Part A of the study

	AZD5634 10 µg (6 participants)	AZD5634 27 µg (7 participants)	AZD5634 81 µg (6 participants)	AZD5634 216 µg (6 participants)	AZD5634 648 µg (6 participants)	AZD5634 1,296 µg (6 participants)	AZD5634 1,692 µg (6 participants)	Placebo (14 participants)
Headache	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (16.7%)	1 (16.7%)	0 (0.0%)	0 (0.0%)	1 (7.1%)
Rash caused by a medical device	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	2 (33.3%)	0 (0.0%)
Atrioventricular block second degree (a heart condition)	1 (16.7%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Muscle twitching	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (16.7%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Nausea	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (16.7%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Vomiting	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (16.7%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Dizziness	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	1 (7.1%)
Ear pain	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	1 (7.1%)
Deep cut	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	1 (7.1%)
Nail infection	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	1 (7.1%)
Viral infection of nose, throat, and sinuses	1 (16.7%)	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%	0 (0.0)%

Most common non-serious medical problems in Part B of the study

	AZD5634 65 µg (Infused) (6 participants)	AZD5634 1,692 µg (Inhaled) (6 participants)
Difficulty breathing	0 (0.0%)	1 (16.7%)
Rash caused by a medical device	1 (16.7%)	0 (0.0%)
Scraped skin	1 (16.7%)	0 (0.0%)

How did AZD5634 act in the body?

Researchers wanted to see how the drug acted in the body. They wanted to know:

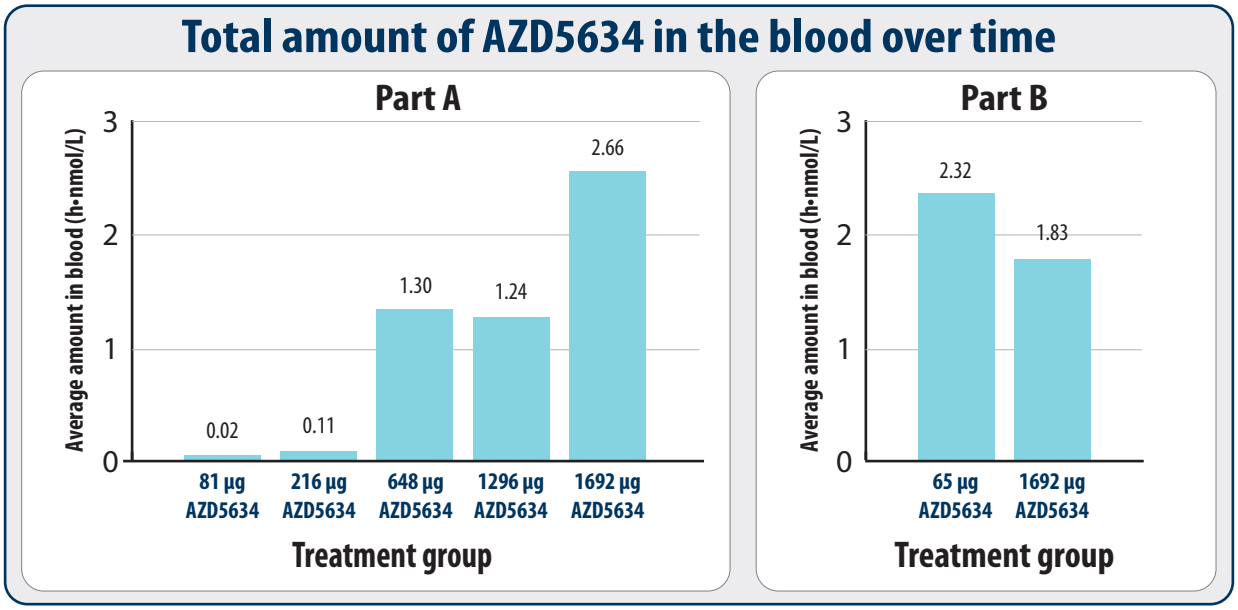
- The total amount of AZD5634 in the blood over time
- The highest amount of AZD5634 in the blood
- How long it took for AZD5634 to reach its highest amount in the blood

Due to very low amounts of AZD5634 in the blood, researchers were not able to measure the results for the participants who got the 10 and 27 µg doses of AZD5634.

Total amount of AZD5634 in the blood over time

Researchers measured the total amount of AZD5634 in the blood over time of participants in nanomoles each hour per liter of blood, or h•nmol/L. This is a widely accepted scientific unit of measurement.

The figures below show this amount for Part A and Part B of the study.

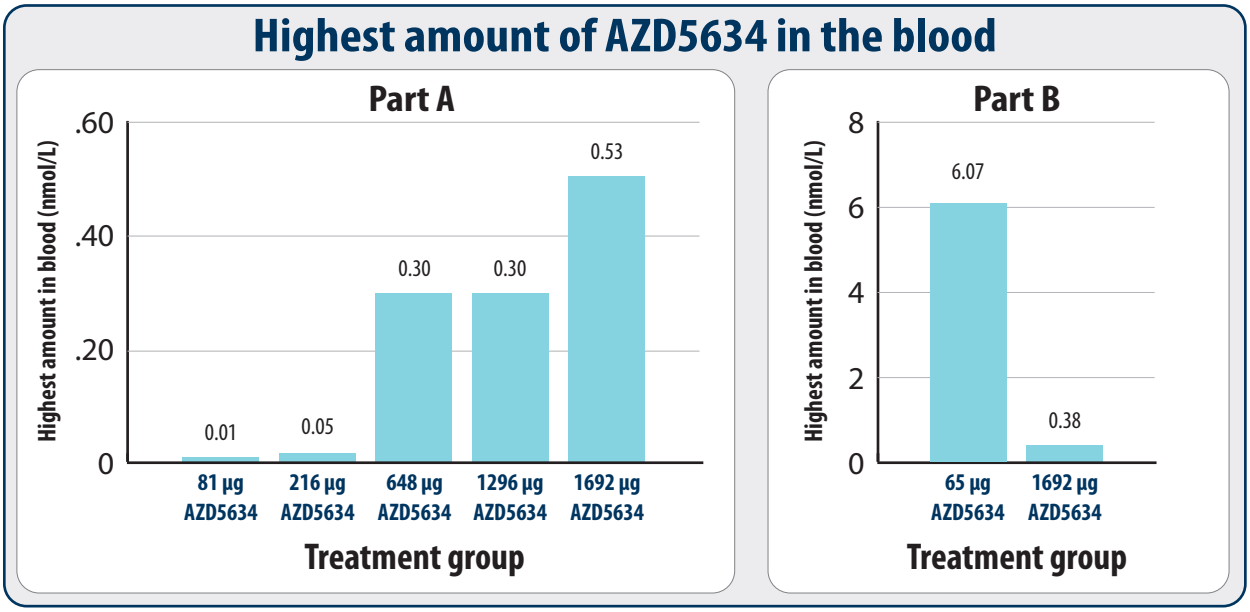


For Part A and Part B, participants who got the higher doses generally had higher total amounts of AZD5634 in the blood over time.

Highest amount of AZD5634 in the blood

Researchers measured the highest amount of AZD5634 in the blood of participants in nanomoles per liter of blood, or nmol/L. This is a widely accepted scientific unit of measurement.

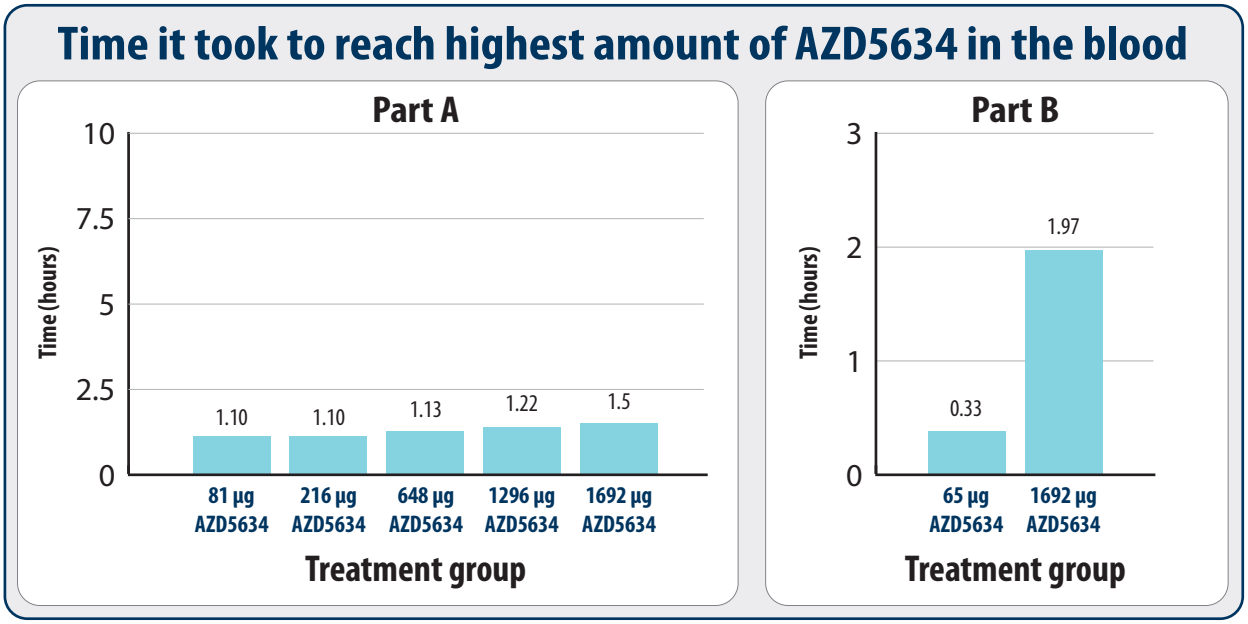
The figures below show this amount for Part A and Part B of the study.



For Part A and Part B, researchers found that there was a relationship between the AZD5634 dose that participants got and the amount of the study drug in the blood.

Time it took for AZD5634 to reach its highest amount in the blood

Researchers measured the time that it took for AZD5634 to reach its highest amount in the blood of participants in hours. The figures below show this time for Part A and Part B of the study.



During Part A, AZD5634 was quickly taken up into the blood after it was breathed in. AZD5634 first started to leave the blood quickly, but then slowed down in leaving the blood, especially in participants who got the higher doses.

During Part B, AZD5634 left the blood very quickly when given through the infusion. During Part B, about 3% of the AZD5634 reached the blood when breathed in.

Where can I learn more about the study?

If you have questions about the results, please speak with the doctor or staff at your study site. You can find more information about your study online at www.clinicaltrials.gov/show/results/NCT02679729.

Official study title: A Phase I, Randomized, Single-Blind, Placebo-Controlled Study to Assess the Safety, Tolerability, and Pharmacokinetics of AZD5634 Following Single-Ascending Inhaled Doses and a Single IV Dose Administration in Healthy Subjects

The phone number for the AstraZeneca Information Center is 1-877-240-9479.

The results presented here are for a single study. Other studies may provide new information or different results. You should not make changes to your therapy based on the results of a single study without first consulting your healthcare professional.

Thank you

It is said that the greatest gift is one which is given anonymously, giving when you do not know whether you will get direct personal benefit.

This is the gift that you have given by taking part in a clinical study. It is a brave and selfless act, one that advances medical knowledge and benefits public health.

Thank you for the gift of your participation in clinical research.

CISCRP
THE CENTER FOR INFORMATION & STUDY ON CLINICAL RESEARCH PARTICIPATION

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 is not involved in recruiting participants for clinical trials, nor is it involved in conducting clinical trials.

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