ForPatients
by Roche

Cystic Fibrosis

A study in healthy volunteers to look at the side effects of a medicine (GDC-6988) – in comparison to a placebo – and the effects of salbutamol

A Phase Ib, Randomized, Double-blind, Placebo-controlled, Single-center Study to Evaluate the Safety and Pharmacokinetics of Multiple Ascending Doses of GDC-6988 With and Without Albuterol Pretreatment in Healthy Adult Subjects

Trial Status	Trial Runs In	Trial Identifier
Completed	1 Countries	2022-000455-36 GB43838

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

Trial Summary:

This clinical trial was done to study a new medicine called, “GDC-6988”, which is being developed for the treatment of patients with “cystic fibrosis.” This study was done to find out if it was safe to give people GDC-6988 treatments using a “Smart Dry Powder Inhaler,” a device for delivering medication to the lungs in the form of a dry powder. This was a Phase 1b, multiple ascending-dose study, that was placebo-controlled, randomized, and double-blind.

Genentech, Inc. (A part of F. Hoffmann-La Roche Ltd., Switzerland)
Sponsor

Phase 1b
Phase

2022-000455-36 GB43838
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Both	18 to 55 years	Yes

Healthy volunteers were enrolled at one study center in the United Kingdom to evaluate the safety and pharmacokinetics of GDC-6988, a medicine (inhaled potentiator) that boosts the activity of a protein in the body (TMEM16A protein). Forty participants completed the multiple ascending-dose, placebo-controlled, randomized, and double-blind Phase 1b study. Based on the lack of serious side effects and the presence of few non-

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serious side effects, researchers thought that people could safely tolerate GDC-6988 at the doses tested.

Background and study aims:

Genentech (the Sponsor) has developed a test medicine to help treat cystic fibrosis; a disease which causes thick mucus to build up within the airways and can cause damage to the lungs and lead to secondary infections which can be life threatening. GDC-6988 (the test medicine) may help the body to hydrate the mucus allowing it to be cleared from the airways naturally.

The purpose of this study is to investigate the test medicine (GDC-6988) given by a test dry powder inhaler at different doses to find out if it is safe and to understand the way your body processes the test medicine. In this study, you will get either GDC-6988 or placebo administered via dry powder inhalation. A placebo looks like a drug but has no active ingredient. GDC-6988 or placebo will be given both with and without salbutamol (also known as albuterol), a drug approved for relieving symptoms of airway obstructive diseases, for example, asthma or chronic obstructive pulmonary disease (COPD). Salbutamol is commonly used to relieve symptoms, such as coughing, wheezing, and feeling breathless. Salbutamol makes it easier to breathe by relaxing the muscles of the airways into the lungs. Salbutamol is being used in this study to assess whether it prevents the side effects seen in some people who are given GDC-6988.

Who can participate?

Healthy volunteers aged between 18 to 55 years of age.

What does the study involve?

Participants will need to be a part of this study for about 70 days. This study will have three parts:

1. A screening visit, where certain tests would be done to determine if the participant is eligible to take part in the study.

2. Treatment period, where eligible participants will be enrolled and will receive an inhaled dose of GDC-6988 or a medication that has no active ingredient (placebo). The treatment (GDC-6988 or placebo) will be decided by chance (like tossing a coin). The participants will have a one in five chance of getting placebo. Treatment will be administered twice a day (about every 12 hours) over about 15 minutes for 14 days, with each dose given using a device called a dry powder inhaler (DPI). On Days 8-14, participants will also receive an inhaled dose of salbutamol given using a DPI, about 15 minutes before each dose of study drug. Participants will be required to check in to the clinic 2 days before receiving the study drug and will be required to stay at the clinic for about 16 nights.

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3. A follow-up period during which participants will have to return to the clinic for two follow-up visits (each lasting about 1-2 hours): one visit about 3 days after the participants check out and another visit about 28 days later.

What are the possible benefits and risks of participating?

Participants will not receive any direct medical benefit, but the information gained from this study may help other people with cystic fibrosis in the future.

Participants may have side effects from the drug (GDC-6988), salbutamol, or procedures used in this study. These can be mild to severe and even life-threatening, and they can vary from person to person. The potential side effects associated with GDC-6988, salbutamol, and other procedures are listed below:

Risks associated with GDC-6988:

GDC-6988 has had limited testing in humans. There are no known side effects of this drug at this point in time. Potential side effects include difficulty in breathing and chest tightness.

Risks associated with Salbutamol:

Side effects associated with salbutamol use include elevated heart rate, shakiness, taste disturbances, and nausea/upset stomach.

Risks associated with the study procedures:

Blood sampling: Drawing blood can cause pain, bruising, or infection where the needle is inserted. Some participants might experience dizziness, fainting, or an upset stomach when their blood is drawn.

There may be a risk in exposing an unborn child to the study drug, and all risks are not known at this time. Women and men must take precautions to avoid exposing an unborn child to the study drug. Participants who are pregnant, become pregnant or are currently breastfeeding cannot take part in this study.

Where is the study run from?

F. Hoffmann-La Roche Ltd (USA)

Who is funding the study?

F. Hoffmann-La Roche Ltd (USA)

Who is the main contact?

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