Plain Language Clinical Study

Summary

This summary reports the results of only one study. Researchers must look at the results of many types of studies to understand if a study medicine works, how it works, and if it is safe to prescribe to patients. The results of this study might be different than the results of other studies that the researchers review.

Sponsor: Pfizer Inc.

Medicine Studied: Osivelotor (formerly PF-07940367 or GBT021601)

Protocol Number: C5351008

Dates of Study: 15 January 2024 to 07 May 2024

Title of this Study: A Study to Learn How Different Products of the Study Medicine Called Osivelotor Are Taken up Into the Blood in Healthy Adults

[A Phase 1, Open-Label, Randomized, Single-Dose, Parallel Design Study to Evaluate Pharmacokinetic Comparability of Two PF-07940367 Film-Coated Tablet Formulations Administered as 150 mg Under Fasted Conditions in Healthy Participants]

Date of this Report: 21 April 2025

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– Thank You –

If you participated in this study, Pfizer, the Sponsor, would like to thank you for your participation.

This summary will describe the study results. Do you have any questions about the study or the results? If so, please contact the doctor or staff at your study site.

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Why was this study done?

What is sickle cell disease?

Sickle cell disease (SCD) is a genetic condition where red blood cells are shaped like a crescent moon (sickle cells). Compared to round (normal) red blood cells, these sickle cells don't carry oxygen well around the body.

SCD leads to problems like anemia (low number of red blood cells), extreme tiredness, attacks of pain, and more frequent infections. SCD lasts a lifetime and can shorten a person's lifespan.

What is osivelotor?

Osivelotor (also called PF-07940367 or GBT021601) is a tablet that is taken by mouth.

Researchers think that osivelotor may help treat SCD. Osivelotor is designed to help red blood cells carry oxygen better throughout the body and stop them from turning into sickle cells.

What was the purpose of this study?

The main goal of this study was to learn how 2 different tablet forms of osivelotor moved through the body and how long it stayed in the blood. After people took osivelotor by mouth, it entered their blood. Researchers wanted to learn how much of the osivelotor stayed in the blood over the next 2 weeks after a single dose.

Researchers tested 2 tablet forms of 150 mg of osivelotor.

- Tablet Form 1
- Tablet Form 2

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Researchers wanted to know:

- **What were the osivelotor blood levels over 2 weeks after taking a single dose of Tablet Form 1 or Tablet Form 2?**
- **What medical problems did participants have during the study?**

What happened during the study?

How was the study done?

Researchers tested 2 different formulations of osivelotor on a group of healthy participants. They wanted to learn more about blood levels of osivelotor in the 2 weeks after taking a single dose.

- Participants took either Tablet Form 1 or Tablet Form 2 of osivelotor

Researchers took blood samples from participants during the study and measured the amount of Tablet Form 1 or Tablet Form 2 of osivelotor. Researchers also checked the participants' health during the study and asked them how they were feeling.

Researchers then compared the results of participants taking Tablet Form 1 to the results of participants taking Tablet Form 2. The participants and researchers both knew who took Tablet Form 1 or Tablet Form 2 of osivelotor. This is known as an "open-label" study. Participants were assigned to each group by chance alone.

Where did this study take place?

The Sponsor ran this study at 1 location in the USA.

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When did this study take place?

It began on 15 January 2024 and ended on 07 May 2024.

Who participated in this study?

The study included healthy participants who were at least 18 years old.

A total of 40 participants took part in this study.

- Twenty (20) participants took Tablet Form 1 of osivelotor, and another 20 participants took Tablet Form 2 of osivelotor.
- A total of 23 men and 17 women participated.
- All participants were between the ages of 29 years and 73 years.

Of the 40 participants who started the study, all 40 finished the study.

How long did the study last?

Study participants were in the study for about 4 months. The entire study took about 4 months to complete. The study was completed as planned.

When the study ended in May 2024, the Sponsor began reviewing the information collected. The Sponsor then created a report of the results. This is a summary of that report.

What were the results of the study?

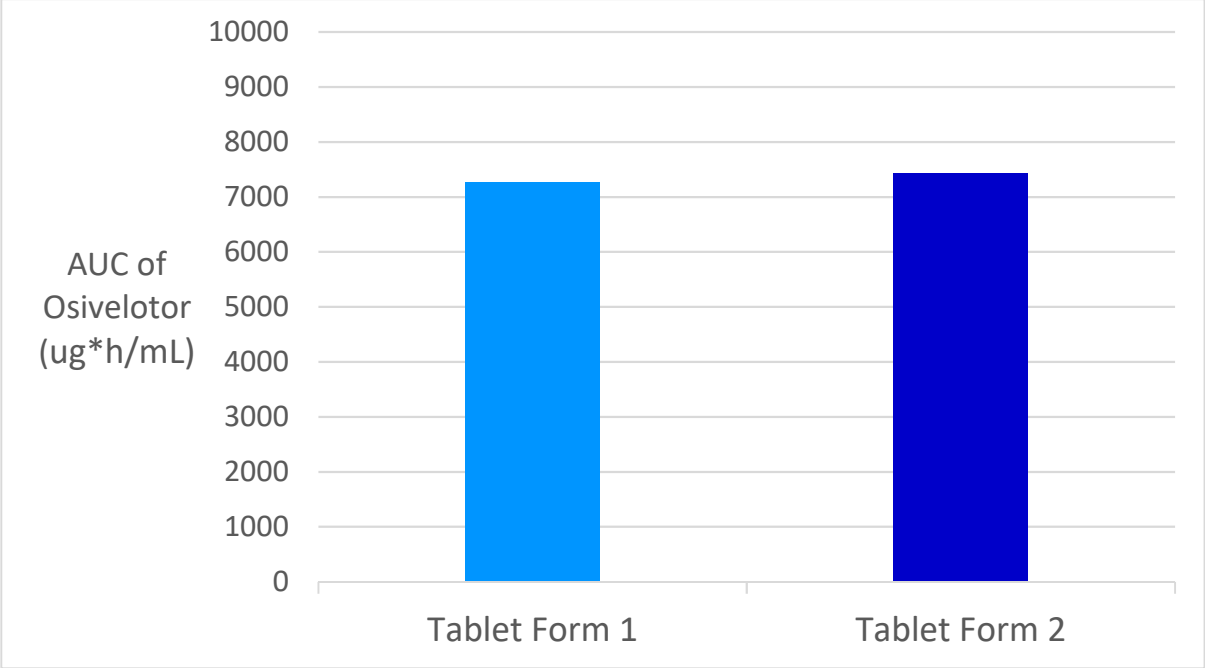
What were the osivelotor blood levels over 2 weeks after taking a single dose of Tablet Form 1 or Tablet Form 2?

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What was the amount of osivelotor in the blood over 2 weeks after participants took 150 mg of Tablet Form 1 or Tablet Form 2?

Area Under the Curve (AUC) is a way to measure how much medicine is in the blood over time. After all the blood samples were taken, the AUC was calculated through 336 hours (2 weeks) after taking the medicine. The average AUC of osivelotor in the blood after participants took 150 mg of Tablet Form 1 or Tablet Form 2 is shown in Figure 1. The amount of medicine in the blood was measured in microgram hours per milliliter, also called ug*h/mL. Researchers considered the difference in the results as minor.

Figure 1 The AUC of osivelotor over 2 weeks



This does not mean that everyone in this study had these results. This is a summary of just some of the main results of this study. Other studies may have different results.

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What medical problems did participants have during the study?

The researchers recorded any medical problems the participants had during the study. Participants could have had medical problems for reasons not related to the study (for example, caused by an unknown underlying disease or by chance). Or, medical problems could also have been caused by a study treatment or by another medicine the participant was taking. Sometimes the cause of a medical problem is unknown. By comparing medical problems across many treatment groups in many studies, doctors try to understand what effects a study medicine might have on a participant.

Four (4) out of 40 participants (10%) in this study had at least 1 medical problem:

- Two (2) participants who took osivelotor Tablet Form 1
- Two (2) participants who took osivelotor Tablet Form 2

None of the participants left the study because of medical problems. All medical problems reported are described below.

Below are instructions on how to read Table 1

Instructions for Understanding Table 1

- The 1st column of Table 1 lists medical problems that were reported during the study. All medical problems reported are listed below.
- The 2nd column tells how many of the 20 participants who took Tablet Form 1 reported each medical problem. Next to this number is the percentage of the 20 participants who took Tablet Form 1 and reported the medical problem.

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- The 3rd column tells how many of the 20 participants who took Tablet Form 2 reported each medical problem. Next to this number is the percentage of the 20 participants who took Tablet Form 2 and reported the medical problem.
- Using these instructions, you can see that 1 out of the 20 participants (5%) who took Tablet Form 1 reported having a headache. A total of 1 out of the 20 participants (5%) who took Tablet Form 2 reported having a headache.

Table 1. Medical problems reported by study participants

Medical Problem	Osivelotor Tablet Form 1 (20 Participants)	Osivelotor Tablet Form 2 (20 Participants)
Headache	1 out of 20 participants (5%)	1 out of 20 participants (5%)
Red, flaky eyelids	1 out of 20 participants (5%)	0
Increased levels of ALT, a liver enzyme	0	1 out of 20 participants (5%)
Increased levels of AST, a liver enzyme	0	1 out of 20 participants (5%)

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Did study participants have any serious medical problems?

A medical problem is considered “serious” when it is life-threatening, needs hospital care, or causes lasting problems.

None of the 40 participants had serious medical problems.

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Where can I learn more about this study?

If you have questions about the results of your study, please speak with the doctor or staff at your study site.

For more details on your study protocol, please visit:

www.pfizer.com/research/research_clinical_trials/trial_results	Use the protocol number **C5351008**
www.clinicaltrials.gov	Use the study identifier **NCT06190561**

Please remember that researchers look at the results of many studies to find out which medicines can work and are safe for patients.

Again, if you participated in this study, **thank you** for volunteering.
We do research to try to find the best ways to help patients, and you helped us to do that!

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