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 (also known as PF-07940367 or GBT021601)

Protocol Number: C5351005 (GBT021601-022)

Dates of Study: 05 January 2023 to 13 February 2025

Title of this Study: A Study of Osivelotor (GBT021601) in Participants With Sickle Cell Disease (SCD)

[An Open-label Extension Study to Evaluate the Long-term Safety of Osivelotor Administered to Participants With Sickle Cell Disease Who Have Participated in an Osivelotor Clinical Trial]

Date of this Report: 23 January 2026

– Thank You –

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

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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.

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 sickled red blood cells do not carry oxygen well around the body.

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

What is osivelotor?

Osivelotor [oh-see-vel-oh-tor] (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 find out if participants with SCD from earlier osivelotor studies can safely take (tolerate) osivelotor for a long time.

Researchers wanted to know:

- **Can participants with SCD tolerate osivelotor for a long time?**
- **What medical problems did participants have during the study?**

This study stopped early because the collected results may not clearly show the benefits and risks of osivelotor. This decision was not due to safety reasons or requests from any health authorities.

What happened during the study?

How was the study done?

Researchers tested osivelotor in participants with SCD who had been in an earlier study of osivelotor to find out if participants could safely take osivelotor for a long time.

Participants took either 100 milligrams (mg), 150 mg, or 200 mg of osivelotor once a day. The dose that participants took in this study was based on the study treatment they took in their earlier osivelotor study.

Participants and researchers knew that all participants took osivelotor in this study. This is called an "open-label" study.

During the study, researchers checked the participants' health, asked how they were feeling, and asked what medications they were taking. About 8 to 12 weeks after the participants' last dose of osivelotor in this study, researchers performed a follow-up check on participants.

Where did this study take place?

The Sponsor ran this study at 8 locations in Nigeria and the United States.

When did this study take place?

It began on 05 January 2023 and ended on 13 February 2025.

Who participated in this study?

The study included participants with SCD who had been in an earlier study of osivelotor. A total of 47 participants joined this study and took at least 1 dose of osivelotor.

- A total of 19 men and 28 women participated.
- All participants were between the ages of 18 years and 61 years.

All participants (100%) did not finish the study. The most common reason was because the Sponsor stopped the study early.

How long did the study last?

Study participants were planned to be in the study for up to 5 years. The entire study ran for about 2 years until it stopped early because the collected results may not clearly show the benefits and risks of osivelotor. The decision to stop the study was not due to safety reasons or requests from any health authorities.

When the study ended in February 2025, 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?

Can participants with SCD tolerate osivelotor for a long time?

To answer this question, researchers looked at all the information collected on the participants' health throughout the study.

Based on the data collected until the study stopped, researchers found that osivelotor was safe and did not increase the risk of having "vaso-occlusive crisis" (VOC) in participants with SCD. VOC, or pain attacks caused by blocked blood vessel, is one of the most common complications of SCD.

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.

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 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.

A total of 39 out of 47 participants (83%) in this study had at least 1 medical problem. The most common medical problems – those reported by more than 10% of total participants – were:

- "**Malaria**," a disease caused by a bite of a mosquito that was infected with a parasite, in 17 out of 47 participants (36%)
- **Urinary tract infection** or UTI in 13 out of 47 participants (28%)
- **Pain** in 8 out of 47 participants (17%)
- **Low number of red blood cells** in 5 out of 47 participants (11%)

One (1) out of 47 participants (2%) left the study because of a medical problem.

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.

A total of 14 out of 47 participants (30%) had serious medical problems. The most common serious medical problem was **low number of red blood cells** in 4 out of 47 participants (9%).

During the study, 1 out of 47 participants (2%) died due to a serious medical problem of "**hemolysis**," a condition where the red blood cells break apart faster than the body can replace them. Researchers do not believe that this serious medical problem was related to osivelotor.

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 **C5351005 (GBT021601-022)**

The full scientific report of this study is available online at:

www.clinicaltrials.gov	Use the study identifier **NCT05632354**
https://euclinicaltrials.eu	Use the study identifier **2023-508768-32-00**

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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