

# 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:** Oxbryta® (voxelotor, also known as PF-06759497 or GBT440)

**Protocol Number:** GBT440-038 (C5341023)

**Dates of Study:** 18 November 2019 to 01 November 2024

**Title of this Study:** Open-Label Extension Study of Voxelotor

[An Open-Label Extension Study of Voxelotor Administered Orally to Participants with Sickle Cell Disease Who Have Participated in Voxelotor Clinical Trials]

**Date of this Report:** 19 December 2025

## – Thank You –

If you or your child 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 or your child's study site.

## Why was this study done?

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### What is sickle cell disease?

Sickle cell disease (SCD) is a condition where red blood cells are shaped like a "sickle" (crescent moon) instead of being round (like a doughnut). These sickled red blood cells break down more quickly and can block blood flow. This makes it harder for the blood to carry oxygen around the body, leading to problems such as anemia (low levels of hemoglobin). Hemoglobin is a protein in the red blood cells that carries oxygen throughout the body.

## What is voxelotor?

Voxelotor (VOX-el-oh-tor), also known as Oxbryta®, is a study medicine given by mouth. In this study, voxelotor was given as a tablet or a dispersible tablet for participants aged 12 years and older and as a suspension for participants aged below 12 years old. A dispersible tablet is a medicine that dissolves in water before drinking it. Suspension is a liquid with solid particles of the medicine (not completely dissolved in the liquid).

Voxelotor is designed to prevent the formation of sickled red blood cells.

## What was the purpose of this study?

The main purpose of this study was to find out if participants from earlier voxelotor studies can safely take voxelotor for a long time.

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### Researchers wanted to know:

- Can participants safely take voxelotor for a long time?
- What medical problems did participants have during the study?

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This study stopped early and was not completed as planned. This was because of safety concerns about voxelotor.

# What happened during the study?

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## How was the study done?

Researchers tested voxelotor in participants who had been in an earlier study of voxelotor to find out if participants could safely take voxelotor for a long time. The participants and researchers knew that all participants were taking voxelotor in this study.

Participants took voxelotor once per day, regardless of the treatment they had taken from the earlier voxelotor study (placebo or voxelotor). A placebo does not have any medicine in it, but it looks just like voxelotor.

- For participants aged **12 years or older**, their dose of voxelotor was 1500 mg.
- For participants aged **under 12 years old**, their dose of voxelotor was based on how much they weighed.

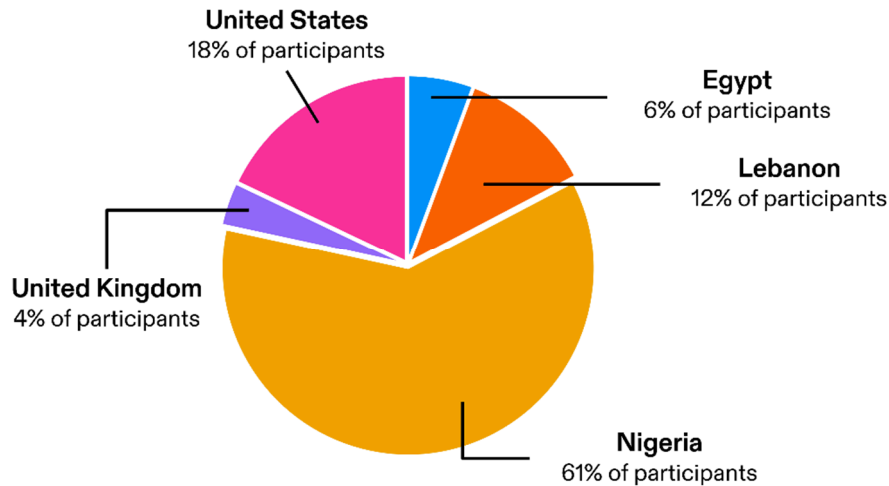
The study planned to allow participants to continue taking voxelotor as long as voxelotor was helping them or until voxelotor was available by prescription where they lived. However, the study ended earlier than planned, and the participants had to stop taking voxelotor. Participants and their parents/caregivers who were still in the study returned to the study site for 1 follow-up visit (within 28 days after taking their last dose).

## Where did this study take place?

The Sponsor ran this study at 26 locations in Egypt, Nigeria, Lebanon, the United Kingdom, and the United States.

Figure 1 below shows the percentage of participants by country.

**Figure 1. Percentage of participants by country**



## When did this study take place?

It started on 18 November 2019 and ended on 01 November 2024.

## Who participated in this study?

The study included participants with SCD who had been in one of earlier studies of voxelotor (GBT440-007, GBT440-032, or GBT440-042).

Of the 162 participants who took part in this study:

- A total of 87 boys/men and 75 girls/women participated.
- All participants were between the ages of 2 and 35 years.

Of the 162 participants who started the study, 25 completed the study. A total of 137 participants did not complete the study. The most common reasons participants stopped the study early were the Sponsor's decision to stop the study early (78% of participants) and participants not following treatment instructions (2% of participants).

## How long did the study last?

Study participants were in the study for different lengths of time, depending on when they started or completed the earlier studies. The study ran for about

5 years before the Sponsor stopped the study early and was not completed as planned.

On 25 September 2024, the Sponsor decided that voxelotor would no longer be available in all markets where it was previously approved. The Sponsor also stopped this study and all ongoing clinical studies on voxelotor. This decision was made at that time because the benefits no longer outweighed the risks.

When the study ended in November 2024, the Sponsor reviewed all 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?

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### Can participants safely take voxelotor for a long time?

To answer this question, researchers looked at any medical problems, including those related to SCD, that participants had.

Overall, participants with SCD who joined this study were able to safely take voxelotor for a long time.

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?

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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.

When participants reported medical problems, the study doctors also noted whether each medical problem was caused by the participant's sickle cell disease (SCD-related) or caused by something else (non-SCD-related).

Figure 2 below shows the number of participants who had at least 1 medical problem in the study.

**Figure 2. How many participants had medical problems?**

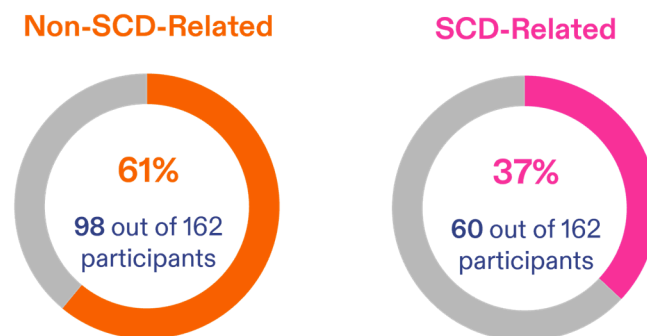
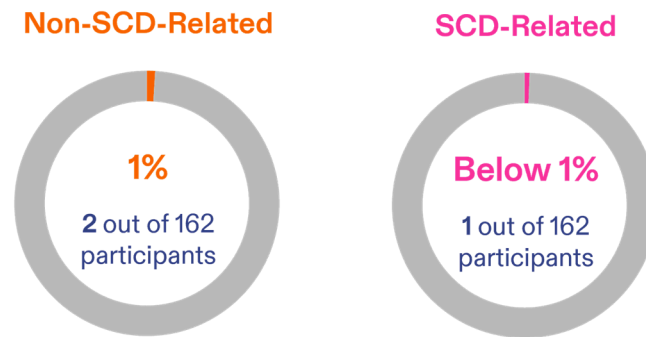


Figure 3 below shows the number of participants who stopped taking voxelotor because of medical problems.

**Figure 3. How many participants stopped taking voxelotor because of medical problems?**



Below are instructions on how to read Table 1 and Table 2.

**Instructions for Understanding Table 1 and Table 2.**

- The **1st** column lists the commonly reported medical problems or serious medical problems in the study. All medical problems or serious medical problems in the tables below were reported by 5% of participants or more. A medical problem is considered “serious” when it is life-threatening, needs hospital care, or causes lasting problems.
- The **2nd** column shows the total number and percentage of participants in the study who reported each medical problem or serious medical problem at least once.

Using these instructions, you can see in **Table 1** below that 56 out of the 162 participants (35%) who took voxelotor reported sickle cell anemia with crisis (sickle cell pain crisis).



People with SCD can develop a sudden pain crisis called “**sickle cell anemia with crisis.**” This happens when the sickled red cells in blood vessels lead to a blockage. As a result, the blood flow and oxygen delivery to areas of the body are prevented, causing painful crises.

**Table 1. Commonly reported medical problems by study participants**

<b>Medical Problem</b>	<b>Voxelotor (162 Participants)</b>
<b>Sickle cell anemia with crisis</b>	56 out of 162 participants (35%)
<b>Fever (pyrexia)</b>	24 out of 162 participants (15%)
<b>Low levels of hemoglobin (anemia)</b>	22 out of 162 participants (14%)
<b>Sudden lung problem causing chest pain and breathing trouble (acute chest syndrome)</b>	16 out of 162 participants (10%)
<b>A disease caused by parasites from infected mosquitoes (malaria)</b>	15 out of 162 participants (9%)
<b>Common cold (upper respiratory tract infection)</b>	13 out of 162 participants (8%)
<b>High levels of potassium in the blood (hyperkalemia)</b>	9 out of 162 participants (6%)
<b>Difficulty passing stool (constipation)</b>	8 out of 162 participants (5%)

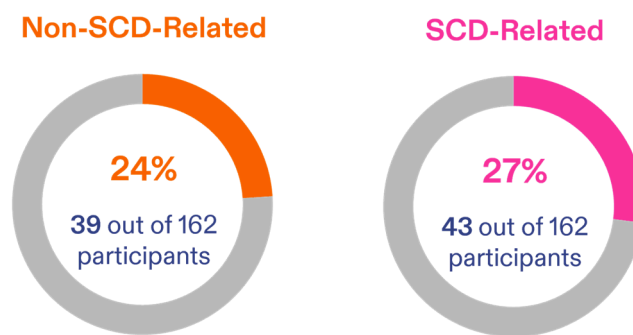
## Did study participants have any serious medical problems?

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

Figure 4 below shows the number of participants who had at least 1 serious medical problem in the study.

**Figure 4.** How many participants had serious medical problems?



**Table 2. Commonly reported serious medical problems by study participants**

<b>Medical Problem</b>	<b>Voxelotor (162 Participants)</b>
<b>Sickle cell anemia with crisis</b>	35 out of 162 participants (22%)
<b>Low levels of hemoglobin (anemia)</b>	16 out of 162 participants (10%)
<b>Sudden lung problem causing chest pain and breathing trouble (acute chest syndrome)</b>	15 out of 162 participants (9%)
<b>A disease caused by parasites from infected mosquitoes (malaria)</b>	8 out of 162 participants (5%)
<b>Fever (pyrexia)</b>	8 out of 162 participants (5%)

One (1) participant died because of a medical problem in the study. The medical problem that led to the participant's death was considered not related to voxelotor.

## Where can I learn more about this study?

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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](http://www.pfizer.com/research/research_clinical_trials/trial_results)

Use the protocol number  
**GBT440-038 (C5341023)**

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

[www.clinicaltrials.gov](http://www.clinicaltrials.gov)

Use the study identifier **NCT04188509**

<https://euclinicaltrials.eu>

Use the study identifier  
**2019-003144-76**

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 or your child 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!

