

# An Open Label, Observational, Prospective Registry of Participants With Sickle Cell Disease (SCD) Treated With Oxbryta® (Voxelotor)® (Voxelotor)

**First published:** 30/09/2024

**Last updated:** 07/04/2025

Study

Finalised

## Administrative details

### EU PAS number

EUPAS1000000170

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### Study ID

1000000170

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### DARWIN EU® study

No

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### Study countries

 United States

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### Study description

The primary objective is to gather long term data on Oxbryta® (Voxelotor)® in a real-world setting. The

following are categories of interest in participants with SCD treated with Oxbryta® (Voxelotor):

- Clinical outcomes, as assessed by clinical and laboratory assessments of hematological parameters and end organ damage, and rate of significant clinical events
- Healthcare resource utilization
- Health-related quality of life (HRQoL), as assessed by participants, parents/caregivers, and clinicians
- Assess the safety and tolerability of Oxbryta® (Voxelotor)®

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## Study status

Finalised

## Research institutions and networks

### Institutions

Pfizer

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

## Contact details

### Study institution contact

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Study contact

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**Primary lead investigator**

Michelle Xu

Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Planned: 02/02/2022

Actual: 02/02/2022

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**Study start date**

Planned: 02/02/2022

Actual: 02/02/2022

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**Data analysis start date**

Planned: 02/02/2022

Actual: 02/02/2022

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**Date of final study report**

Planned: 30/04/2030

Actual: 06/03/2025

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

GBT, Pfizer

## Study protocol

[GBT440-4R2 \(C5341019\) Registry Protocol \(Prospective\) GBT template 3 February 2021\\_FINAL.pdf](#) (770.73 KB)

[No Pedigree GBT440-4R2 \(C5341019\)\\_Non-Interventional Study Protocol \\_Final\\_v1.0\\_10Oct2023.pdf](#) (1.6 MB)

## Regulatory

### **Was the study required by a regulatory body?**

No

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### **Is the study required by a Risk Management Plan (RMP)?**

Not applicable

## Other study registration identification numbers and links

NCT04930445

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Disease /health condition

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**Study topic, other:**

Observational study designed to evaluate the effect of Oxbryta® (Voxelotor) in individuals with SCD.

**Study type:**

Non-interventional study

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**Scope of the study:**

Other

**If 'other', further details on the scope of the study**

Register observational

**Data collection methods:**

Primary data collection

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**Study design:**

Any participant who is currently taking Oxbryta® (Voxelotor) or has been prescribed and will initiate treatment with Oxbryta® (Voxelotor), is eligible to participate. Eligible participants will receive treatment with Oxbryta® (Voxelotor) as prescribed by their physician, as part of their usual care.

**Main study objective:**

The primary objective is to gather long term data on Oxbryta® (Voxelotor)® in a real-world setting. The following are categories of interest in participants with SCD treated with Oxbryta® (Voxelotor):

- Clinical outcomes, as assessed by clinical and laboratory assessments of hematological parameters and end organ damage, and rate of significant

clinical events

- Healthcare resource utilization Health-related quality of life (HRQoL), as assessed by participants, parents/caregivers, and clinicians
- Assess the safety and tolerability of Oxbryta® (Voxelotor)®

## Study Design

### **Non-interventional study design**

Other

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### **Non-interventional study design, other**

Any participant who is currently taking Oxbryta® (Voxelotor) or has been prescribed and will initiate treatment with Oxbryta® (Voxelotor), is eligible to participate. Eligible participants will receive treatment with Oxbryta® (Voxelotor) as prescribed by their physician, as part of their usual care. Participants will be treated and evaluated per SOC and at the physician's discretion.

## Study drug and medical condition

### **Medicinal product name**

OXBRYTA

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### **Study drug International non-proprietary name (INN) or common name**

VOXELOTOR

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### **Anatomical Therapeutic Chemical (ATC) code**

(B06AX03) voxelotor

## **Medical condition to be studied**

Sickle cell disease

## **Population studied**

### **Short description of the study population**

Participants who meet all the following criteria will be eligible for enrollment:

1. Willing and able to provide written informed consent (aged  $\geq 18$  years), parental/guardian consent and participant assent (aged  $\geq 12$  to  $<18$  years) per local regulations, or pediatric participants (aged 4 to  $<12$  years) with parental/guardian consent per Institutional Review Board (IRB) policy and requirements, consistent with ICH guidelines
  2. Male or female participants with documented diagnosis of sickle cell disease (all genotypes)
  3. Undergoing treatment with Oxbryta® (Voxelotor) according to the Oxbryta® (Voxelotor) USPI
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### **Age groups**

- **Paediatric Population ( $< 18$  years)**

- Preterm newborn infants (0 - 27 days)
- Term newborn infants (0 - 27 days)
- Children (2 to  $< 12$  years)
- Adolescents (12 to  $< 18$  years)

- **Adult and elderly population ( $\geq 18$  years)**

- Adults (18 to  $< 65$  years)
  - Adults (18 to  $< 46$  years)
  - Adults (46 to  $< 65$  years)

- Elderly ( $\geq 65$  years)
    - Adults (65 to  $< 75$  years)
    - Adults (75 to  $< 85$  years)
    - Adults (85 years and over)
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### **Estimated number of subjects**

500

## Documents

### **Study report**

[C5341019 PROSPECT GBT440-4R2 Final NI Clinical Study Report\\_Redacted.pdf](#)

(10.16 MB)

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

### **Data sources (types)**

[Disease registry](#)

## Use of a Common Data Model (CDM)

## **CDM mapping**

No

## Data quality specifications

### **Check conformance**

Unknown

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No