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

Study ID

1000000170

DARWIN EU® study

No

Study countries

☐ United States

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)®

Study status

Finalised

Research institutions and networks

Institutions

Pfizer

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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

Study start date

Planned: 02/02/2022

Actual: 02/02/2022

Data analysis start date

Planned: 02/02/2022

Actual: 02/02/2022

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

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

Study topic, other:

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

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

Register observational

Data collection methods:

Primary data collection

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

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

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

VOXELOTOR

Anatomical Therapeutic Chemical (ATC) code

(B06AX03) voxelotor

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 ≥ 18 years), parental/guardian consent and participant assent (aged ≥ 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

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 (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)

- Elderly (≥ 65 years)
  - Adults (65 to < 75 years)
  - Adults (75 to < 85 years)
  - Adults (85 years and over)

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

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No