A Retrospective Data Collection and Analysis Study of Patients With Sickle Cell Disease (SCD) Who Have Been Treated With Oxbryta® (Voxelotor)

First published: 14/10/2024

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Study Ongoing

Administrative details

EU PAS number

EUPAS1000000334

Study ID

1000000334

DARWIN EU® study

No

Study countries

United States

Study description

This is a retrospective data collection and analysis study. Patients will have received treatment with Oxbryta as prescribed by their physician at the approved dose per local prescribing information, as part of their usual care.

Primary
The following are categories of interest in patients with SCD treated with Oxbryta:
• Clinical outcomes, as assessed by clinical and laboratory assessments of hematological parameters and end organ damage, and incidence of significant clinical events
• Healthcare resource utilization
• Health-related quality of life (HRQoL), as assessed by patient-reported outcome (PRO) measures and clinician-reported outcomes (ClinRO)

Safety: The safety objective is to assess the safety and tolerability of Oxbryta.

Study status
Ongoing

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: 26/01/2021

Actual: 26/01/2021

Study start date

Planned: 31/03/2021

Actual: 20/03/2021

Date of final study report

Planned: 14/10/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

GBT440-4R1 Registry Protocol (Retrospective)_FINAL redacted.pdf (266.6 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Data collection methods:

Secondary use of data

Study design:

This is a multicenter, retrospective data collection and analysis study to characterize health outcomes in approximately 300 patients with SCD who have been treated with Oxbryta as part of their usual care. Data will be collected at approximately 10 sites in the US.

Main study objective:

Primary

The following are categories of interest in patients with SCD treated with Oxbryta:

• Clinical outcomes, as assessed by clinical and laboratory assessments of hematological parameters and end organ damage, and incidence of significant clinical events

• Healthcare resource utilization

• Health-related quality of life (HRQoL), as assessed by patient-reported outcome (PRO) measures and clinician-reported outcomes (ClinRO)

Safety

The safety objective is to assess the safety and tolerability of Oxbryta.

Study Design

Non-interventional study design

Case-control

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

1. Willing and able to provide written informed consent (ages >= 18 years) or parental/guardian consent and patient assent (age <18 years), as required by the IRB, institution, or per local regulations

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

Estimated number of subjects

300

Study design details

Setting

This is a multicenter, retrospective data collection and analysis study to characterize health outcomes in approximately 300 patients with SCD who have been treated with Oxbryta as part of their usual care.

Any patient with SCD who received Oxbryta treatment for at least 2 weeks as part of their usual care according to the Oxbryta US Prescribing Information (USPI) is eligible to participate.

Patients will be introduced to the study by their health care team and will sign the informed consent form (ICF) to allow their data to be collected and used for the study, if required by the Institutional Review Board (IRB), institution, or per local regulations.

Only data that are available from the patient's medical records and other secondary data sources will be collected. Study data from 1 year before and up to 1 year after the first dose of Oxbryta will be entered in case report forms (CRFs) via an electronic data capture (EDC) system by the study staff.

Outcomes

• Change from pre-Oxbryta treatment period in the following hematologic parameters corresponding to treatment with Oxbryta:
o Hb
o Hemolysis measures, including % reticulocytes, absolute reticulocytes, bilirubin (total, direct, and indirect)
o Measures of iron overload, including ferritin, iron, total iron binding capacity (TIBC), T2-weighted magnetic resonance imaging (T2*MRI), and liver biopsy
• Change from pre-Oxbryta treatment period in renal function, as measured by the following:
o Albuminuria (urine albumin/creatinine ratio [ACR])
o Hemoglobinuria (urine dipstick positive for blood +1 or greater and :: 2 RBC by high power field)
o Serum cystatin C
o Estimated glomerular filtration rat (eGFR) calculated using the Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI) equation
• Incidence of significant SCD-related clinical events, such as vaso-occlusive crisis (VOC), acute chest syndrome (ACS), priapism, cerebral infarcts, transient ischemic attack (TIA), leg ulcers, and measures of cardiac function and pulmonary hypertension (PH)
• Treatment initiation or modification of SCD-related medications (e.g., hydroxyurea, crizanlizumab, L-glutamine, opioids [in daily morphine equivalents], iron chelating agents, erythropoiesis-stimulating agents [ESAs], nonsteroidal anti-inflammatory drugs [NSAIDs], folic acid, and penicillin)
• Change from pre-Oxbryta treatment period in healthcare resource utilization: incidence of unplanned clinic visits, emergency department (ED) visits, hospitalizations (including total length of stay and time in intensive care unit [ICU], if applicable), acute and chronic RBC transfusions, home oxygen supplementation, and renal dialysis
• Change from pre-Oxbryta treatment period in the following HRQoL measures:

o Patient-Reported Outcomes Measurement Information System (PROMIS)

o Patient Global Impression of Change (PGIC)

o Clinical Global Impression of Change (CGIC)

Data analysis plan

After informed consent/assent has been obtained (if required by the institution or IRB), the data listed below that are available at or around the timepoints indicated in the schedule of activities (SOA) in Appendix 1 will be collected and entered into the EDC by study personnel at the study site. Data collection will include only those data that are recorded in the patient's medical records and other secondary data sources, up to the timepoint when the data is extracted. Study data that are not available in medical records or other secondary data sources will not be solicited from patients.

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 source(s), other

Rutgers Cancer Institute of New Jersey

Brigham and Women's Hospital

Montefiore Medical Center

Central Michigan University/Children's Hospital of Michigan

UT Southwestern Medical Center

Levine Cancer Institute

Duke University Hospital

University of Connecticut Health

University of Texas Health Science Center at Houston

Data sources (types)

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Yes

Check completeness

Yes

Check stability

Yes

Check logical consistency

Yes

Data characterisation

Data characterisation conducted

Unknown