Effectiveness of voxelotor in individuals with sickle cell disease and a history of red blood cell transfusions: A non-interventional, retrospective cohort study using real-world data in the United States

First published: 31/07/2024

Last updated: 07/04/2025

Study Finalised

Administrative details

EU PAS number

EUPAS1000000225

Study ID

1000000225

DARWIN EU® study

No

Study countries

☐ United States

Study description

After identifying the populations of interest using CEM on age and calendar time, the distribution of baseline covariates will be evaluated in the 365 days prior to index.

Continuous variables will be described using mean values with standard deviations and median values with interquartile range; categorical variables will be described as the number of patients and percent.

For variables derived from diagnoses, procedures, and prescription codes, patients are assumed to have experienced the event of interest if the relevant code(s) are found among their claim records.

Otherwise, it is assumed that the patient did not experience the event, thus resulting in no missing data for these variables

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Study status

Finalised

Research institutions and networks

Institutions

Aetion

Spain

First published: 24/11/2022

Last updated: 16/07/2024

Institution | Other | ENCePP partner

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Carmine Colavecchia

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 13/03/2023

Actual: 31/03/2023

Study start date

Planned: 01/12/2024

Actual: 08/01/2025

Data analysis start date

Planned: 01/12/2024

Actual: 08/01/2025

Date of interim report, if expected

Planned: 01/02/2025

Actual: 03/03/2025

Date of final study report

Planned: 08/02/2025

Actual: 03/03/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

C5341060_Pfizer_Oxbryta Reduction in Transfusion Study Protocol_PASS_V2.0_23Dec2024 _Redacted.pdf (766.55 KB)

C5341060_Pfizer_Oxbryta Reduction in Transfusion Study_Protocol_V2.1_09Jan2025_Redacted.pdf (19.48 MB)

Regulatory

Was the study required by a regulatory body?

Unknown

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

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This is a non-interventional, retrospective cohort study of individuals with SCD using a US real-world dataset composed of linked closed claims, proprietary voxelotor prescription claims, and laboratory data.
Propensity score (PS) methods will be utilized to adjust for measured confounding covariat

Main study objective:

Compare the change in red blood cell (RBC) transfusions per patient per year (PPPY) from baseline (one year prior to index date) to follow-up (up to one year after index date).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

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

The study population will include patients with SCD between the ages of 12 and 85 years of age who are enrolled in a health insurance plan continuously during the baseline period.

Age groups

Adolescents (12 to < 18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Special population of interest

Other

Special population of interest, other

Individuals with sickle cell disease and a history of red blood cell transfusions

Estimated number of subjects

5000

Study design details

Setting

The study population will include patients with SCD between the ages of 12 and 85 years who are enrolled in a health insurance plan (commercial, Medicare, Medicaid) continuously during the 365 days prior to and including the index date (baseline).

The index selection period is November 25, 2019 (date of FDA accelerated approval for voxelotor) through one year prior to the end of the study period.

The exposed group will include patients with a new prescription claim for voxelotor (no prior use 365 days prior to index date), while the control group will include matched patients who do not have a prescription claim for voxelotor. Non-voxelotor controls will include those receiving no SCD modifying therapies as well as those receiving non-voxelotor SCD treatments to be reflective of clinical practice.

Patients who are receiving chronic transfusion therapy for stroke prevention will be excluded from this analysis.

Both the exposed and control groups may be treated with other SOC treatments such as HU.

Data analysis plan

Detailed methodology for summary and statistical analyses of data collected in this study will be documented in the SAP, which will be dated, filed, and maintained by Pfizer.
The SAP may modify the plans outlined in the protocol; any major modifications of primary endpoint definitions or their analyses would be reflected in a protocol amendment.

Documents

Study report

C5341060 -Interventional-Low-Interventional Study Type Study Report Redacted.pdf (10.44 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 source(s), other

Optum Electronic Health Records and Claims Data, Komodo’s Healthcare Map (KHM) database, Claritas prescription data, Quest Diagnostics lab data

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

Yes

Data characterisation moment

after data extraction