241501: Prospective and retrospective, non-interventional study to evaluate the safety and effectiveness of Obizur in real-life practice

First published: 20/11/2016

Last updated: 22/02/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/48205

EU PAS number

EUPAS16055

Study ID

48205

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Netherlands
- [ ] Poland
- [ ] United States

Study description

The study addresses the safety, utilisation and effectiveness of Obizur in the treatment of bleeding episodes in real-life clinical practice in Europe and the United States.

Study status

Finalised

Research institutions and networks

Institutions

Shire

First published: 01/02/2024

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Institution

Multiple centres: 36 centres are involved in the study

Contact details

Study institution contact

Study Contact Shire

Study contact

clinicaltransparency@shire.com

Primary lead investigator

Study Contact Shire

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 21/03/2016

Actual: 21/03/2016

Study start date

Planned: 03/01/2017

Actual: 14/12/2016

Data analysis start date

Planned: 02/07/2021

Actual: 30/07/2021

Date of final study report
Planned: 01/06/2022

Actual: 01/06/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Baxalta Innovations GmbH, now part of Shire

Study protocol

241501-protocol-original-redact.pdf(1.56 MB)

241501-protocol-amend-3-redact.pdf(1.66 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The primary objective of the study is to document the safety of subjects treated with Obizur in real-life clinical practice by recording any AEs, including but not limited to: - Hypersensitivity reactions - Thromboembolic events - Dose dispensing medication errors

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Post-authorisation, prospective and retrospective, single cohort, uncontrolled, multi-centre European and US study

Study drug and medical condition

Name of medicine

OBIZUR

Medical condition to be studied

Acquired haemophilia

Population studied

Short description of the study population

A subject must be prescribed Obizur for the treatment of a bleeding episode by a physician, independent of and prior to the decision to enrol the subject in the study.

Inclusion Criteria

Subjects who meet ALL of the following criteria are eligible for this study:

1. Adult subject or legal authorised representative is willing to provide informed consent, unless informed consent is not required (e.g. subjects who are deceased),
as local regulations allow.
2. Subject is being treated or was treated with Obizur in routine clinical practice

Exclusion Criteria

Subjects who meet ANY of the following criteria are not eligible for this study:

1. Subject has participated in a clinical study involving a medicinal product or device within 30 days prior to enrolment or is scheduled to participate in a clinical study involving a medicinal product or device at study entry
2. Subject has known anaphylactic reactions to the active substance, hamster protein, or to any of the excipients of Obizur listed in the SPC /PL
- The following list of excipients can be found in the SPC/PL:

o Powder:
- Polysorbate 80
- Sodium chloride
- Calcium chloride dihydrate
- Sucrose
- Tris Base
- Tris HCl
- Tri-sodium citrate dihydrate

o Solvent:
- Sterilised water for injections

3. US Subject who has participated in the post-marketing study, NCT2610127

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

50

Study design details

Outcomes

The primary endpoint is safety, which will be assessed by adverse event (AE)/ serious adverse event (SAE) frequency, seriousness, severity and outcome (subject recovered/ recovered with sequelae, not recovered/ fatal). Particular attention will be given to the following adverse event of special interest (AESI): hypersensitivity reactions, thromboembolic events and dose dispensing medication errors, - Immunogenicity: newly recognised anti-pFVIII inhibitors or increase in titre of anti-pFVIII inhibitors and evolution of titre over time - Clinical characteristics of the treated subject population - Overall effectiveness assessment for resolution of bleeding determined as either bleeding stopped or did not stop - Time and dosage administered to achieve bleeding control

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Data analysis plan

Categorical variables will be summarised by absolute and relative frequencies (number of valid and missing observations and percentages). Continuous variables will be summarised by descriptive statistics (number of valid and missing observations, mean, standard deviation, median, interquartile range, minimum, and maximum). Two-sided 95% CI will be provided for the main statistical estimator.

Documents

Study results

241501-clinical-study-report-redact.pdf(196.35 KB)

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective and retrospective patient-based data collection

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