A POST AUTHORIZATION SAFETY SURVEILLANCE REGISTRY IN PREVIOUSLY UNTREATED PATIENTS WITH SEVERE HEMOPHILIA A IN USUAL CARE SETTINGS

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Study Planned

Administrative details

EU PAS number

EUPAS5235

Study ID

6917

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ Bulgaria

- [ ] Croatia
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Italy
- [ ] Morocco
- [ ] Netherlands
- [ ] Portugal
- [ ] Romania
- [ ] Serbia
- [ ] Spain
- [ ] Türkiye
- [ ] United Kingdom

Study description

The study will evaluate the safety of ReFacto AF in the usual care setting in previously untreated patients with severe hemophilia A. Primary objective is to evaluate clinically significant FVIII inhibitor development in two groups of subjects. Secondary objective is overall safety of ReFacto AF in this Registry population.This Registry will be conducted to observe subjects using ReFacto AF at hemophilia treatment centers in Europe and other regions where ReFacto AF is commercially available.

Study status

Planned

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

Rendo Pablo

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 14/10/2013

Actual: 14/10/2013

Study start date

Planned: 15/07/2014

Date of final study report

Planned: 27/01/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer Inc

Regulatory

Was the study required by a regulatory body?

Yes

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Main study objective:

The primary objective of this Registry is to evaluate clinically significant FVIII inhibitor development in hemophilia A subjects who were PUPs and ≤6 years of age upon initiating treatment with ReFacto AF (first group) as well as in hemophilia A subjects who had attained 50 to 149 EDs, are ≤8 years of age, and successfully completed participation in another study (B1831006) (second group).

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Primary data collection, voluntary prospective Registry

Study drug and medical condition

Medicinal product name

REFACTO AF

Additional medical condition(s)

SEVERE HEMOPHILIA A

Population studied

Age groups

- Preterm newborn infants (0 – 27 days)
- Term newborn infants (0 – 27 days)
- Infants and toddlers (28 days – 23 months)
- Children (2 to < 12 years)

Estimated number of subjects

100

Study design details

Outcomes

Clinically significant positive FVIII inhibitor, which is defined in this Registry as:a. 2 positive FVIII inhibitor results (using either Bethesda Inhibitor Assay or the Nijmegen Modification of the Bethesda Inhibitor Assay) within a 4 week period. Occurrence of adverse events (AEs).

Data analysis plan

The results of this study will be presented using descriptive statistics. The primary analysis will be performed on all subjects who receive at least one dose of ReFacto AF during participation in this Registry. The two groups: prospectively identified PUPs (with 0 to 7 ReFacto AF EDs at the time of Registry enrollment, Group A subjects), and subjects who completed study B1831006 (with at least 50 EDs and “completed” B1831006 and not “withdrawn or discontinued”) will be analyzed independently with respect to hemophilia history, adverse events, inhibitor development, demography, and disposition. The primary safety outcome, the proportion of subjects who develop clinically significant inhibitors, will be reported. Secondary safety outcomes, the frequency of adverse events and serious adverse events, will be summarized. The reasons for discontinuation from the Registry or from treatment with

ReFacto AF therapy will be described.

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)

Other

Data sources (types), other

Prospective 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