Prospective, multinational, non-interventional post-authorisation study to document the long-term immunogenicity, safety, and efficacy of Human-cl rhFVIII (simoctocog alfa) in patients with haemophilia A treated in routine clinical practice (GENA-99)

First published: 24/11/2015

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Study Finalised

Administrative details

EU PAS number

EUPAS7905

Study ID

43614

DARWIN EU® study

No

Study countries

 Argentina

 Belarus

 Czechia

 Ecuador

 France

 Guatemala

 Italy

 Lithuania

 Norway

 Portugal

 Slovakia

 United Kingdom

 United States

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Study description

The number of patients enrolled in the pre-authorisation clinical studies with Human-cl rhFVIII was 135 (see Section 2.2.1), which is considered adequate to provide relevant information on general safety aspects and to demonstrate the efficacy of Human-cl rhFVIII in terms of its ability to restore factor VIII levels and stop or prevent bleeding. However, data from pre-authorisation studies are insufficient to estimate all aspects of therapy with FVIII products, especially with respect to immunogenicity (EMA/CHMP/BPWP/144533/2009). Therefore, to estimate the likelihood of rare side effects, such as the occurrence of FVIII inhibitors, and to bridge between the outcome from clinical trials and long-term clinical use, the purpose of this study is to assess the long-term immunogenicity, safety, and efficacy of Human-cl rhFVIII in patients with haemophilia A treated in routine clinical practice. Specifically, the study is designed to meet the requirements for post-authorisation studies as outlined in ‘Guideline on the clinical investigation of recombinant and human plasma-

derived factor VIII products’ by the European Medicines Agency (EMA), EMA/CHMP/BPWP/144533/2009

Study status

Finalised

Research institutions and networks

Institutions

Multiple centres: 38 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Kate Khair

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 26/06/2014

Actual: 22/07/2014

Study start date
Planned: 01/02/2016

Actual: 21/01/2016

Date of final study report
Planned: 31/03/2020

Actual: 26/03/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Octapharma AG

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Other

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

To collect clinical data to ensure the consistency, in the long-term, between the outcome from pre-authorisation clinical studies (in 135 previously treated patients)

Data collection methods:

Primary data collection

Main study objective:

•To assess the long-term immunogenicity and safety of Human-cl rhFVIII in treating or preventing bleeding episodes (BEs) in patients with haemophilia A• To assess the long-term efficacy of Human-cl rhFVIII in treating or preventing BEs in patients with haemophilia A

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Non randomised observational study, Post-authorisation study

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(B02BD02) coagulation factor VIII

coagulation factor VIII

Medical condition to be studied

Haemophilia A without inhibitors

Population studied

Short description of the study population

Male patients with haemophilia A without inhibitors treated in routine clinical practice receiving rhFVIII (simoctocog alfa)
At least 100 patients should have severe haemophilia A. Approx. 60 patients should be < 12 years of age; also, at least 10 patients should be aged between 14–18 years. Patients with severe haemophilia A after successful immune tolerance induction (ITI) can also be included; the proportion of ITI patients should not exceed 25% of the entire cohort.

Age groups

- Infants and toddlers (28 days – 23 months)
- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- 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)

Special population of interest

Other

Special population of interest, other

Hemophilia A patients

Estimated number of subjects

200

Study design details

Outcomes

long-term immunogenicity and safety of Human-cl rhFVIII,long-term efficacy of Human-cl rhFVIII

Data analysis plan

The data recorded in the CRF will be analysed using descriptive statistical methods. An analysis plan detailing the analyses to be performed will be prepared by the study statistician and approved by the Sponsor before the start of the study.

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