Non-interventional study of long term treatment with Haemoctin SDH (Biotest NIS-016)

First published: 08/06/2016

Last updated: 02/07/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS13728

Study ID

27437

DARWIN EU® study

No

Study countries

Australia

Germany

Hungary

Study description

Haemoctin SDH is a factor VIII (FVIII) preparation derived from human plasma purified by chromatography. Haemoctin SDH is approved for prevention and treatment of innate and acquired factor VIII deficiencies. Also, patients with a FVIII inhibitor can be treated with Haemoctin SDH. Details are given in the SPC of Haemoctin SDH. The stabilization of the FVIII molecule is carried out by the natural carrier protein von Willebrand factor. There is no need for the addition of auxiliary stabilizers such as sucrose or human serum albumin. Hemophilia A is an inherited, chronic bleeding disorder and patients have to be treated lifelong with FVIII concentrates. Most children and adolescents are treated prophylactically in industrialized countries. Prophylaxis has the goal to avoid bleedings, in order to guarantee the patient a high quality of life (QoL). Patients who have started in childhood with the prophylaxis, this treatment is extended in adulthood. Prophylactic treatment consists of regular FVIII applications, usually three times a week. With this study long-term data will be generated. Unique longterm data obtained form some patients in a previous study with Haemoctin SDH over up to 22 year can be extended with this study. This NIS allows adopting the documentation to the current guidance for observational studies and adjusted focus of the objectives.

Study status

Finalised

Research institutions and networks

Institutions

Biotest

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Christoph Königs

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/02/2016

Actual: 12/04/2016

Study start date

Planned: 01/03/2016

Actual: 01/06/2016

Data analysis start date

Planned: 31/12/2021

Actual: 01/01/2023

Date of final study report
Planned: 30/09/2023

Actual: 08/12/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biotest AG

Study protocol

2021-02-18 Haemoctin NIS Observation Plan_V2 clean.pdf (192.41 KB)

Regulatory

Was the study required by a regulatory body?

No

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

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

With this NIS long-term data for the effectiveness in bleedingprevention and on QoL will be generated.The following questions will be examined:· What are the factors influencing the risk of bleeding over thetime of treatment?· What are the factors influencing the risk to develop FVIIIinhibitors during treatment with Haemoctin SDH?· Can these inhibitors be further characterized?

Study Design

Non-interventional study design

Other

Study drug and medical condition

Medicinal product name, other

Haemoctin SDH

Medical condition to be studied

Factor VIII deficiency

Population studied

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)
- Term newborn infants (0 – 27 days)
- 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

Immunocompromised

Estimated number of subjects

150

Study design details

Outcomes

Annual bleeding rate defined as episodes per year in patients with Haemoctin SDH treatment, differentiated by prophylaxis and on demand Treatment. · AE and subsequent suspected ADR (AE assessed as causally related with Haemoctin treatment) · AE with bleeding = AE of special interest (AESI) with extended bleeding documentation, for e.g. if the duration and severity of the bleeding is within the situation as expected or unexpected · characterization of FVIII inhibitors to Haemoctin SDH · QoL determined with the EQ-5D

Data analysis plan

All analyses will be performed in an exploratory sense. Data will be analyzed using descriptive statistics. For continuous variables, mean, standard deviation, minimum, maximum, median, 25% and 75% percentiles will be presented. Qualitative and categorical variables will be presented by means of absolute and relative frequencies. A medical evaluation of the findings will be performed. Details of analysis will be described in a statistical analysis plan.

Documents

Study results

BT13218_CSR_Final_v1.0_20231208_Redacted.pdf (2.9 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 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