An Open-Label, Multicenter Evaluation of the Long-Term Safety and Efficacy of Recombinant Human Coagulation Factor VIII Fusion Protein (rFVIIIFc) in the Prevention and Treatment of Bleeding Episodes in Previously Treated Subjects With Hemophilia A

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Ethical review Approved WMO **Status** Recruitment stopped

Health condition type Coagulopathies and bleeding diatheses (excl thrombocytopenic)

Study type Interventional

Summary

ID

NL-OMON44048

Source

ToetsingOnline

Brief title

ASPIRE 8HA01EXT

Condition

• Coagulopathies and bleeding diatheses (excl thrombocytopenic)

Synonym

Clotting deficiency, clotting factor VIII deficiency

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Research involving

Human

Sponsors and support

Primary sponsor: Biogen

Source(s) of monetary or material Support: Pharmaceutical Industry

Intervention

Keyword: Bleeding Episodes, Extension, Hemophilia A, rFVIIIFc

Outcome measures

Primary outcome

Occurence of inhibitor development

Secondary outcome

Secondary:

The annualized number of bleeding episodes (spontaneous and traumatic) per

subject

The annualized number of spontaneous joint bleeding episodes per subject

The total number of days of exposure per subject per year

The consumption of rFVIIIFc as total dose per kg per subject per year

Physician*s global assessment of subject's response to his treatment using a

4-point scale

Subject*s assessment of response to treatment of bleeding episodes using a

4-point scale

Study description

Background summary

To evaluate the long-term safety of rFVIIIFc for prevention and on-demand treatment of bleeding episodes in subjects with hemophilia A, and to allow subjects from the A-LONG pivotal study (997HA301), the pediatric study (8HA02PED), or any other rFVIIIFc study to continue treatment with rFVIIIFc. Subjects will continue in this study for up to 4 years or until rFVIIIFc is commercially available in the applicable participating country. Subjects will follow a tailored or personalized prophylactic regiment chosen by the study doctor. Once the child is 12 years old or older also other options can be chosed such as once-weekly modified prophylaxis or on-demand regimen based on the clinical profile of the subject and by the trough or peak (recovery) values, if needed, as observed in the preceding study. Subjects are allowed to change treatment regimens (for example, from prophylaxis, including modified prophylaxis, to on-demand, or from on-demand to prophylaxis) during the study.

Study objective

An Open-Label, Multicenter Evaluation of the Long-Term Safety and Efficacy of Recombinant Human Coagulation Factor VIII Fusion Protein (rFVIIIFc) in the Prevention and Treatment of Bleeding Episodes in Previously Treated Subjects With Hemophilia A.

The primary objective of the study is to evaluate the long-term safety of rFVIIIFc in subjects with hemophilia A.

Study design

This is an open-label, multicenter, long-term study of intravenous (IV) administration of rFVIIIFc in previously treated patients with hemophilia A, who have completed the A-LONG study (997HA301), the pediatric study (8HA02PED), or any other trial with rFVIIIFc. Treatment will be administered in tailored or personalized prophylaxis, once-weekly modified prophylaxis or on-demand regimens.

Intervention

Study drug: Recombinant Human Coagulation Factor VIII Fusion Protein (rFVIIIFc) per IV administration

Study burden and risks

Benefit: A possible benefit may be the need for less frequent injections to manage his hemophilia. What we learn from this study may lead to improved understanding of how the study drug works when taken for several years by people with hemophilia A.

Risks: The examinations done during the study may cause discomfort. Taking

blood from a vein may cause bruising,

localized bleeding, infection, faintness, and a small amount of pain from the needle puncture.

Side effects known to occur with other Factor VIII products may also occur with the study drug. Side effects that have

occurred with other FVIII products include symptoms of itching, rash, local reactions at the injection site (such as burning

and temporary redness), unusual taste in the mouth, fever, headache, discomfort or swelling around the vein from which

blood was drawn, bleeding/bruising at the injection site, and Factor VIII inhibitor formation.

Contacts

Public

Biogen

Innovation House, Norden Road 70 Maidenhead, Berkshire SL6 4AY GB

Scientific

Biogen

Innovation House, Norden Road 70 Maidenhead, Berkshire SL6 4AY GB

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age

Children (2-11 years)

Inclusion criteria

- 1. Ability to understand the purpose and risks of the study and provide signed and dated informed consent and authorization to use protected health information (PHI) in accordance with national and local subject privacy regulations. Parental or guardian consent is required for subjects who are less than 18 years of age or unable to give consent, or as applicable per local laws. Subjects who are less than 18 years of age may provide assent in addition to the parental/guardian consent, if appropriate.
- 2. Subjects who have completed the studies 997HA301, 8HA02PED, or other Phase 3 studies with rFVIIIFc.

Exclusion criteria

- 1. Confirmed positive high-titer inhibitor test (*5.00 BU/mL)
- 2. Current enrollement in any other study.
- 3. Inability to comply with study requirements.
- 4. Other unspecified reasons that, in the opinion of the Investigator or Biogen Idec, make the subject unsuitable for enrollment.

Study design

Design

Study phase: 3

Study type: Interventional

Masking: Open (masking not used)

Control: Uncontrolled

Primary purpose: Prevention

Recruitment

NL

Recruitment status: Recruitment stopped

Start date (anticipated): 07-10-2013

Enrollment: 6

Type: Actual

Medical products/devices used

Product type: Medicine

Brand name: ELOCTA

Generic name: EFMOROCTOCOG ALFA

Registration: Yes - NL intended use

Ethics review

Approved WMO

Date: 10-04-2013

Application type: First submission

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 17-07-2013

Application type: First submission

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 18-09-2013

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 31-10-2013

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 27-02-2014

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 17-03-2014

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 19-02-2015

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 20-03-2015

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 07-08-2015

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 18-08-2015

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Approved WMO

Date: 25-05-2016

Application type: Amendment

Review commission: METC Universitair Medisch Centrum Groningen (Groningen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register ID

EudraCT EUCTR2011-003072-37-NL

CCMO NL43600.042.13
Other NTC01454739