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

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 unsuitalbe 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