

Clinical study to investigate the long-term safety and efficacy of human cell line recombinant Factor VIII (human-cl rhFVIII) in previously treated patients with severe haemophilia A

Submission date 09/11/2009	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
		<input type="checkbox"/> Protocol
Registration date 16/11/2009	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
Last Edited 04/01/2012	Condition category Haematological Disorders	<input type="checkbox"/> Individual participant data
		<input type="checkbox"/> Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
2009-014422-41

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers

GENA-04

Study information

Scientific Title

Study objectives

Investigation of the long-term immunogenic potential of human cell line recombinant Factor VIII (human-cl rhFVIII).

As of 03/01/2012, the anticipated end date was corrected from 01/01/2012 to 01/07/2011.

Ethics approval required

Old ethics approval format

Ethics approval(s)

Ethics Committee at the Federal Supervision Service for Public Health and Social Affairs approved on the 9th September 2009 (ref: "Case EC-37284)

Study design

Prospective open-label clinical trial

Primary study design

Interventional

Secondary study design

Non randomised controlled trial

Study setting(s)

Hospital

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use the contact details below to request patient information material

Health condition(s) or problem(s) studied

Severe haemophilia A

Interventions

All patients will be treated in accordance with their needs until the product is registered and launched in the country of conductance. There are no further interventions planned beside the three-monthly control of FVIII recovery and inhibitor development.

Intervention Type

Drug

Phase

Not Applicable

Drug/device/biological/vaccine name(s)

Human cell line recombinant Factor VIII (human-cl rhFVIII)

Primary outcome measure

Immunogenicity: Inhibitor activity will be determined by the modified Bethesda assay (Nijmegen modification) at three months intervals until study completion. At the same time-points the anti-rhFVIII antibodies will be measured.

Secondary outcome measures

1. Clinical tolerability: assessed by monitoring vital signs (blood pressure, heart rate, respiratory rate, and body temperature will be assessed at pre-defined time-points)
2. Laboratory parameters:
 - 2.1. Haematological parameters - red blood cell count, white blood cell count, haemoglobin, haematocrit, and platelet count
 - 2.2. ALAT, ASAT, serum creatinine
3. Adverse events (AEs)
4. Prophylactic treatment: the frequency of bleeds under prophylactic treatment will be calculated. Study drug consumption data (FVIII IU/kg per month, per year) per subject and in total will be evaluated.
5. Treatment of bleeding episodes: efficacy assessment at the end of each BE
6. In-vivo recovery: calculated from the FVIII:C plasma levels measured before infusion and peak level obtained in the 30 or 60 minutes post-infusion sample and the actual potency of Human-cl rhFVIII. FVIII:C in the product and in plasma will be measured both by the chromogenic (CHR) and the one-stage (OS) assay.

Overall study start date

01/11/2009

Completion date

01/07/2011

Eligibility**Key inclusion criteria**

1. Must have severe haemophilia A (FVIII:C less than 1%; historical value as documented in subject records)
2. Aged greater than 18 years and less than 65 years, male only
3. Body weight 45 kg to 110 kg
4. Previously treated with human-cl rhFVIII (within study GENA-09)
6. Negative for human immunodeficiency virus (HIV) or respective viral load less than 200 particles/ μ L
7. Freely given written informed consent

Participant type(s)

Patient

Age group

Adult

Lower age limit

18 Years

Sex

Male

Target number of participants

22 (Recruitment completed)

Key exclusion criteria

1. Other coagulation disorder than haemophilia A
2. Present or past FVIII inhibitor activity (greater than 0.6 BU)
3. Severe liver or kidney disease (alanine aminotransferase [ALAT] and aspartate aminotransferase [ASAT] levels greater than 5 times of upper limit of normal, creatinine greater than 120 $\mu\text{mol/L}$)
4. Receiving or scheduled to receive immuno-modulating drugs (other than anti-retroviral chemotherapy) such as alpha-interferon, prednisone (equivalent to greater than 10 mg/day), or similar drugs
5. Participation in another clinical study currently or during the past month, except GENA-09

Date of first enrolment

01/11/2009

Date of final enrolment

01/07/2011

Locations

Countries of recruitment

Austria

Russian Federation

Study participating centre

Oberlaaerstrasse 235

Vienna

Austria

1100

Sponsor information

Organisation

Octapharma AG (Switzerland)

Sponsor details

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Sponsor type

Industry

Website

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Funder(s)**Funder type**

Industry

Funder Name

Octapharma AG (Switzerland)

Results and Publications**Publication and dissemination plan**

Not provided at time of registration

Intention to publish date**Individual participant data (IPD) sharing plan****IPD sharing plan summary**

Not provided at time of registration