ISRCTN90038418 https://doi.org/10.1186/ISRCTN90038418

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	[ ] Prospectively registered [ ] Protocol [ ] Statistical analysis plan [ ] Results [ ] Individual participant data [ ] Record updated in last year
Registration date 16/11/2009	**Overall study status** Completed	
Last Edited 04/01/2012	**Condition category** Haematological Disorders	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Clinical Trials Information System (CTIS)
2009-014422-41

Protocol serial number
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

Study type(s)
Treatment

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(s)
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.

Key secondary outcome(s))
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.

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/µL
7. Freely given written informed consent

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Sex
Male

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 µmol/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
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Sponsor information

Organisation
Octapharma AG (Switzerland)

ROR
https://ror.org/002k5fe57

Funder(s)

Funder type
Industry

Funder Name
Octapharma AG (Switzerland)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes