Clinical Trial Protocol
Iranian Registry of Clinical Trials

12 Jul 2026

Inhibitor Development in Previously Untreated Patients (PUPs) or Minimally Blood Component-Treated Patients (MBCTPs) when Exposed to plasma-derived von Willebrand Factor-Containing Factor VIII (VWF/FVIII) Concentrates and to Recombinant Factor VIII (rFVIII) Concentrates

Protocol summary

Summary

Patients enrolled in the trial will be treated for the first 50 EDs or for the first 3 years from enrolment, whichever comes first, with either a single plasma-derived FVIII concentrate containing von Willebrand factor or a single rFVIII concentrate as assigned by the randomisation. Treatment with FVIII products other than that initially prescribed will represent a reason for withdrawal. The therapeutic regimen for on-demand treatment will be at the discretion of the physician, even though a dose of 25-40 IU/Kg is recommended, to repeat until complete resolution of the episode, or 40 IU/Kg at bleeding occurrence, followed by 20 IU/Kg at 24 and 72 hours. The dosage for prophylaxis will be at the discretion of the physician. The suggested regimen for standard prophylaxis is 25-40 IU/Kg, 3-4 times a week. For the sake of the analysis, prophylaxis is defined as at least 2 infusions weekly on non-consecutive days with 20 IU/Kg or more. Any other regular treatment will be defined as modified prophylaxis. Patients will be monitored for inhibitor development by Bethesda assay with Nijmegen modification locally and at the central laboratory in case of treatment failure or every 3 to 4 EDs for the first 20 EDs and every 10 EDs thereafter, or every 3 months, whichever come first, and at any time inhibitor development would be clinically suspected. Patients on prophylaxis will be tested every 2 weeks. In case of suspect inhibitor development, inhibitor levels must be assessed locally (a second aliquot will be stored in the refrigerator) and confirmed both locally and at the central laboratory within 14 days after the first positive test and every month for the following 6 months.

General information

Acronym

SIPPET

IRCT registration information

IRCT registration number: **IRCT201104106161N1**
Registration date: **2011-04-30, 1390/02/10**
Registration timing: **registered_while_recruiting**

Last update:
Update count: **0**

Registration date

2011-04-30, 1390/02/10

Registrant information

Name

Bahman Vasseghi

Name of organization / entity

Clinart

Country

United Arab Emirates

Phone

0097144370551

Email address

bahman.vasseghi@clinart.net

Recruitment status

Recruitment complete

Funding source

Fondazione Angelo Bianchi Bonomi- Italy

Expected recruitment start date

2009-04-01, 1388/01/12

Expected recruitment end date

2014-03-31, 1393/01/11

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title
Inhibitor Development in Previously Untreated Patients (PUPs) or Minimally Blood Component-Treated Patients (MBCTPs) when Exposed to plasma-derived von Willebrand Factor-Containing Factor VIII (VWF/FVIII) Concentrates and to Recombinant Factor VIII (rFVIII) Concentrates

Public title
Survey of Inhibitors in Plasma-Product Exposed Toddlers

Purpose
Prevention

Inclusion/Exclusion criteria
Inclusion criteria: 1-Male subjects; 2-Any ethnicity; 3-Age <6 years; 4-Severe haemophilia A (FVIII:C <1%), as confirmed by the central laboratory; 5-Previously untreated (0 EDs to any FVIII concentrate or blood products) or minimally treated (<5 EDs) with blood components, namely whole blood, fresh frozen plasma, packed red blood cells, platelets or cryoprecipitate; 6-Negative inhibitor measurement at both local and central laboratory at screening; 7-Ability to comply with study requirements; 8-Signed informed consent of legal tutors. Exclusion criteria: 1-Plasma FVIII level ≥1%, as assayed at the central laboratory; 2-Previous history of FVIII inhibitor; 3-Other congenital or acquired bleeding defects; 4-Concomitant congenital or acquired immunodeficiency; 5-Concomitant treatment with systemic immunosuppressive drugs; 6-Concomitant treatment with any investigational drug.

Age
To **6 years** old

Gender
Male

Phase
4

Groups that have been masked
No information

Sample size
Target sample size: **300**

Randomization (investigator's opinion)
Randomized

Randomization description

Blinding (investigator's opinion)
Not blinded

Blinding description

Placebo
Not used

Assignment
Parallel

Other design features
Non-Profit, Investigator-initiated, Multicentre and international

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee
National Ethics Committee

Street address
Deputy of research and technology- ministry of health

City
Tehran

Postal code

Approval date
2010-02-02, 1388/11/13

Ethics committee reference number
790 - ص88رپ

Health conditions studied

1

Description of health condition studied
Hemophilia A

ICD-10 code
D66

ICD-10 code description
Hereditary factor VIII deficiency

Primary outcomes

1

Description
To assess the immunogenicity of plasma derived VWF/FVIII and rFVIII concentrates

Timepoint
in the first 50 EDs or in the first 3 years from enrolment

Method of measurement
by determining the frequency of inhibitor development

Secondary outcomes

1

Description
To evaluate modality of occurrence of inhibitors (number of EDs, titre at onset, etc)

Timepoint
in the first 50 EDs or in the first 3 years from enrolment, whichever comes first

Method of measurement
record and analysis of characteristics during the treatment course

Intervention groups

1

Description
Recombinant Factor VIII concentrate

Category
Treatment - Drugs

2

Description
plasma-derived Factor VIII concentrate
Category
Treatment - Drugs

Recruitment centers

1

Recruitment center
Name of recruitment center
comprehensive care centre for children with hemophilia
Full name of responsible person
Dr. Peyman Eshghi
Street address
Mofid children hospital
City
Tehran

2

Recruitment center
Name of recruitment center
Hemophilia treatment centre
Full name of responsible person
Dr. Mehran Karimi
Street address
Shahid Dastgheib Hospital
City
Shiraz

Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Fondazione Angelo Bianchi Bonomi
Full name of responsible person
Barbara Bianchi Bonomi
Street address
Piazza Castello, 2 20121
City
Milano
Grant name
Grant code / Reference number
Is the source of funding the same sponsor organization/entity?
Yes
Title of funding source
Fondazione Angelo Bianchi Bonomi
Proportion provided by this source
100
Public or private sector
empty
Domestic or foreign origin
empty
Category of foreign source of funding
empty
Country of origin
Type of organization providing the funding
empty

Person responsible for general inquiries

Contact
Name of organization / entity
Clinart
Full name of responsible person
Dr. Bahman Vasseghi
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Study manager-Iran/MD
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Person responsible for scientific inquiries

Contact
Name of organization / entity
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Person responsible for updating data

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Web page address

Sharing plan

Deidentified Individual Participant Data Set (IPD)

empty

Study Protocol

empty

Statistical Analysis Plan

empty

Informed Consent Form

empty

Clinical Study Report

empty

Analytic Code

empty

Data Dictionary

empty