

Clinical Trial Protocol

Iranian Registry of Clinical Trials

16 Sep 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 $\geq 1\%$, 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

رپ 88 ص - 790

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

Position

Study manager-Iran/MD

Other areas of specialty/work

Street address

205, building 72, Healthcare city

City

Dubai

Province

Dubai

Postal code

Phone

0097144370551

Fax

Email

bahman.vasseghi@clinart.net

Web page address

Person responsible for scientific inquiries

Contact

Name of organization / entity

Department of Medicine, University of Milan

Full name of responsible person

Prof. Pier Mannuccio Mannucci

Position

study coordinator

Other areas of specialty/work

Street address

Via Pace, 9 - 20122

City

Milano

Province

Milan

Postal code

Phone

0039 02 55035421

Fax

Email

piermannuccio.mannucci@unimi.it

Web page address

Person responsible for updating data

Contact

Name of organization / entity

Clinart

Full name of responsible person

Dr. Bahman Vasseghi

Position

Study manager-Iran/MD

Other areas of specialty/work**Street address**

205, Building 72, Healthcare city

City

Dubai

Province

Dubai

Postal code**Phone**

0097144370551

Fax**Email**

bahman.vasseghi@clinart.net

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