The Dynamic interplay between bleeding phenotype and baseline factor level in moderate and mild hemophilia A and B

Published: 13-09-2017
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The primary aim of this project is to analyze the association between factor VIII and factor IX plasma levels and the bleeding phenotype.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Coagulopathies and bleeding diatheses (excl thrombocytopenic)
Study type	Observational invasive

Summary

ID

NL-OMON55705

Source
ToetsingOnline

Brief title
Dynamo

Condition

- Coagulopathies and bleeding diatheses (excl thrombocytopenic)

Synonym
bleeding disorder, Hemophilia A and B

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: NovoNordisk

Intervention

Keyword: Baseline factor level, Bleeding phenotype, Hemophilia A, Hemophilia B

Outcome measures

Primary outcome

Part 1 Cohort study

Data will be collected on demographics, physical activity level, musculoskeletal status, genotype, measured factor levels (both one stage and chromogenic), the nature and duration of all bleeding episodes, type of treatment and treatment response. Central laboratory assays for FVIII or FIX levels will be performed.

Secondary outcome

Part 2: Detailed substudy

A subset of 50 patients aged 24 years or older (50 with moderate/mild hemophilia A) will be investigated in more detail by data collection including analysis of a) physical joint status and function; b) 3Tesla MRI imaging and c) biomarkers for joint damage.

Study description

Background summary

There are large inter-individual differences in the bleeding pattern of patients with moderate or mild hemophilia. The major determinant of bleeding phenotype is the FVIII or FIX plasma level. In hemophilia A, studies addressing the association between FVIII plasma level and the clinical bleeding phenotype yield conflicting results. In hemophilia B such studies have not yet been performed.

Study objective

The primary aim of this project is to analyze the association between factor VIII and factor IX plasma levels and the bleeding phenotype.

Study design

Observational cohort study

Study burden and risks

For part 1, Cohort study, the burden and risks of participation is the withdrawal of one blood sample for central measurement. All data will be collected from the medical files.
For part 2, Detailed substudy, the burden and risks of participation consist of a physical examination addressing the joint status and function, MRI imaging of the ankle and knee joint and withdrawal of blood samples.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age

Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- Moderate or mild hemophilia A (FVIII:C 0.02*0.35 IU/mL) or hemophilia B (FIX:0.02*0.35 IU/ml)
- 12-55 years old
Extra for substudy:
- 24 years of age or older

Exclusion criteria

- Other clotting disorder
- Participation in another trial with an investigational product
- Comorbidity affecting the musculoskeletal status
- Clinically relevant inhibitor status at present or in the past
- Hemophilia B Leyden
- Use of anticoagulants

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	16-01-2018
Enrollment:	190
Type:	Actual

Ethics review

Approved WMO Date:	13-09-2017
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	19-12-2017
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	22-01-2018
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	26-03-2018
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	23-10-2018
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	29-05-2019
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	24-07-2019
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	22-03-2021
Application type:	Amendment
Review commission:	METC Amsterdam UMC

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
CCMO	NL61564.018.17