Von Willebrand disease in the Netherlands - Prospective study (WiN-Pro)

Published: 18-09-2018
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Our primary aim is to investigate the current bleeding tendency of patients with VWD.

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

Summary

ID

NL-OMON55831

Source
ToetsingOnline

Brief title
WiN-Pro

Condition

- Coagulopathies and bleeding diatheses (excl thrombocytopenic)
- Blood and lymphatic system disorders congenital

Synonym
Von Willebrand disease, VWD

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: CSL Behring,Shire,Stichting Haemophilia (Dutch Haemophilia Foundation)

Intervention

Keyword: Bleeding disorder, Nationwide study, von Willebrand disease, von Willebrand factor

Outcome measures

Primary outcome

The major-bleeding rate: bleeding that require blood transfusion, desmopressine

or factor concentrates or bleeding that lead to death.

Secondary outcome

Secundary outcomes are the minor-bleeding rate (all bleeding that do not fulfil

the criteria for major-bleeding), bleeding during surgery, Von Willebrand

factor levels and the ISTH-BAT score.

Study description

Background summary

Von Willebrand disease (VWD) is the most common inherited bleeding disorder, and is characterized by a defective platelet adhesion and aggregation. VWD is caused by a reduced (type 1), an abnormal function (type 2) or a complete absence (type 3) of von Willebrand factor (VWF).
The diagnosis of VWD is based on the evaluation of the personal bleeding history and family history, followed by detailed laboratory evaluation. To assess the bleeding phenotype, the International Society for Thrombosis and Hemostasis Bleeding Assessment Tool (ISTH-BAT) is recommended as bleeding score. The laboratory evaluation of VWD consists out of measurement of VWF antigen (VWF:Ag), and VWF activity: VWF-mediated platelet agglutination (VWF:RCo) and the binding of VWF to collagen (VWF:CB), and the coagulant activity of Factor VIII (FVIII:C).
In recent years large retrospective cohort studies have provided valuable insights on the clinical presentation, bleeding phenotype, quality of life, diagnostics, genetics and treatment of patients with VWD. All large retrospective cohort studies have assessed the bleeding phenotype of patients with VWD using bleeding scores or retrospective questionnaires. Bleeding scores calculate the sum of all bleeding episodes during lifetime. Therefore, they do not provide information on the change of bleeding tendency. If a patient had a

period in his or her lifetime in which he or she had many bleeding episodes, then the bleeding score is high. Though, the patient could have had those bleeds 30 years ago and did not have a bleeding episode since then. Therefore, bleeding scores do not provide information on the current bleeding phenotype of VWD patients, and prospective studies are needed to investigate the current bleeding tendency of patients with VWD.

Study objective

Our primary aim is to investigate the current bleeding tendency of patients with VWD.

Study design

We will conduct a nationwide, multi-center, observational, prospective cohort study in all VWD patients in the Netherlands.

This study has 2 important elements; patients will visit their own hemophilia treatment center for study inclusion and patients will be followed-up for a period of 2 years, during which they will fill in online questionnaires each 3 months.

Study burden and risks

This study has an observational character. The only interventions in this study are the questionnaires, a blood drawal at study inclusion, a blood pressure measurement and a capillary nail fold measurement. We consider the risks of this study to be negligible and the burden to be minimal. The total burden of this study is approximately 2 to 3 hours during a period of 2 years.

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)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

- Historically lowest VWF:Ag and/or VWF:Act and/or VWF:CB <= 0.30 IU/mL and/or FVIII:C <= 0.40 IU/mL.
- Treatment at a Hemophilia treatment center in the Netherlands
- All types of VWD
- All ages

Exclusion criteria

- Other bleeding disorders present

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	23-07-2019
Enrollment:	1300
Type:	Actual

Ethics review

Approved WMO Date:	18-09-2018
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	09-12-2021
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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
Other	Identificatienummer volgt
CCMO	NL62238.078.18