Prospective data collection on patients with rare bleeding disorders (PRO-RBDD)

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Ethical review	Approved WMO
Status	Recruiting
Health condition type	Other condition
Study type	Observational invasive

Summary

ID

NL-OMON44839

Source
ToetsingOnline

Brief title
Prospective registry of rare bleeding disorders (PRO-RBDD)

Condition

- Other condition

Synonym
bleeding disorder

Health condition

Bloedstollingsafwijkingen

Research involving
Human

Sponsors and support

Primary sponsor: Angelo Bianchi Bonomi Hemophilia and Trombosis Center - University of Milan and Luigi Villa Foundation
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: Hemostasis, PRO-RBDD, Rare bleeding disorder, Registry

Outcome measures

Primary outcome

Laboratory phenotype and genotype, type / site / number of spontaneous

bleedings or triggered episodes, treatment use, efficacy, and safety.

Secondary outcome

Not applicable.

Study description

Background summary

Rare Bleeding Disorders (RBDs), like fibrinogen, factor II, factor V, factor V+VIII, factor VII, factor X, factor XI and factor XIII deficiencies, are relatively rare in Western Countries (1 : 0,5-2 million). Inadequate therapy is associated with severe musculoskeletal handicaps and sometimes early death. The development of guidelines and treatment recommendations for these disorders have been hampered by a lack of well-designed collection of data and accurate statistical analysis.
The International Database on RBDs (*RBDD*) has obtained retrospective information that helped immensely to improve the knowledge on frequency and distribution of each coagulation defect. However, despite the valuable findings obtained, there is still a gap on annual incidence of either disorders or the bleeding episodes as well as on the minimum coagulant activity level that is able to prevent spontaneous and trauma related bleedings and to provide an adequate haemostasis.
The new registry *PRO-RBDD* will collect prospective data on prevalence, bleeding frequency in relation to clotting factor level and genotype, and management and consumption of treatment products in patients affected with RBD.

These data will be analysed to set up the optimal prophylaxis scheme.

Study objective

The main objective is to improve treatment with clotting factor concentrate of patients affected by RBDs by collecting and analysing data on the patients genotype, laboratory phenotype and clinical severity.
Secondary objectives are to gain information on incidence and prevalence of RBDs and bleedings, on consumption of treatment products, on the diagnostic value of several assays, on the relationship between genetic defects and clinical/laboratory phenotype and to set up facilities to control genetic defects through prenatal diagnosis.

Study design

Prospective registration study.

Study burden and risks

There are no considerable risks or benefits for the patients. The burden is minimal: over five years there will be 11 data entry points per patient. Baseline data collection will be performed at the clinic during a visit including blood sampling (maximum volume 20 ml). Follow-up consists of initial screening contact by telephone and personal check-up in case of report of (suspected) bleeding episodes or other medical events. Also children will be included in this study, since the population of patients having RBDs is very small.

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

Severe, moderate or mild congenital deficiency of one of the following coagulation factors: fibrinogen (factor I); prothrombin (factor II); factor V; factor V+VIII; factor VII; factor X; factor XI; factor XIII.

Exclusion criteria

Vitamin K deficiency or other anti-coagulants.

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	22-08-2013
Enrollment:	40
Type:	Actual

Ethics review

Approved WMO Date:	11-07-2013
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	08-09-2014
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	25-05-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	09-10-2017
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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	NL44517.091.13