Stop study.

No registrations found.

Ethical review	Positive opinion
Status	Pending
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON25201

Source
NTR

Health condition

haemophilia, prophylaxis, adherence, bleeds, arthropathy.

hemofilie, profylaxe, therapietrouw, bloedingen, arthropathie.

Sponsors and support

Primary sponsor: University Medical Center Utrecht (UMC Utrecht)
Source(s) of monetary or material Support: Novo Nordisk

Intervention

Outcome measures

Primary outcome

1. Joint status according to Haemophilia Joint Health Score (HJHS) and Pettersson score;

2. Annual number of joint bleeds;

3. Figure 8 walk test.

Secondary outcome

1. Clotting factor consumption;

2. Orthopaedic surgery;

3. Hospital admissions in the third decade of life;

4. Major soft tissue bleeds.

Study description

Background summary

Primary prophylaxis was designed as a life-long replacement therapy to prevent bleeds and maintain joint function in patients with severe haemophilia. Maintaining this treatment is a heavy burden for the patient, and treatment is likely to include periods of reduced adherence. Information on the consequences of tapering or discontinuing prophylaxis, and knowledge of which patients may try this without taking irresponsible risks is vital in the support of this life-long treatment.

The present project will assess patient-initiated changes in long-term prophylaxis in the second and third decade. By measuring outcome, safety of discontinuing or tapering prophylaxis, will be assessed. Additionally a prognostic model aims to identify clinical parameters as predictors of successful discontinuation and tapering.

This information will provide an evidence base for day-to-day issues in treating young adults with severe haemophilia, help to individualise prophylactic treatment, as well as increase cost-effectiveness of prophylaxis.

Study objective

Is there a group of severe haemophilia patients that can reduce or stop prophylaxis in their 2nd and 3rd decade without an increased risk of bleeding and joint damage?

Study design

Retrospective collection of treatment history and cross-sectional assessment of outcome measured from 2011 until 2013.

Intervention

N/A

Contacts

Public
PO Box 85500
Kathelijn Fischer
Van Creveldkliniek
Room: C01.425
Utrecht 3508 GA
The Netherlands
+31 (0)88 7558450
Scientific
PO Box 85500
Kathelijn Fischer
Van Creveldkliniek
Room: C01.425
Utrecht 3508 GA
The Netherlands
+31 (0)88 7558450

Eligibility criteria

Inclusion criteria

1. Severe haemophilia A (factor VIII < 0.01 IU/ml);

2. Born between January 1st 1970 and January 1st 1988;

3. Registered at the haemophilia treatment centres in Utrecht (NL), Århus and Copenhagen (DK).

Exclusion criteria

1. History of inhibitory activity of more than 5 BU at any time or less than 5 BU for more than one year;

2. Inadequate access to treatment during the first years of life (i.e. no access to unlimited replacement therapy during the first 4 years of life, e.g. in case of immigration);

3. Insufficient follow up or insufficient data;

4. Other pathology influencing bleeding pattern.

Study design

Design

Study type:	Observational non invasive
Intervention model:	Parallel
Allocation:	Non-randomized controlled trial
Masking:	Single blinded (masking used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-11-2011
Enrollment:	90
Type:	Anticipated

Ethics review

Positive opinion

Date:	10-10-2011
Application type:	First submission

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
NTR-new	NL2950
NTR-old	NTR3098
Other	WHO UTN : U1111-1121-7069
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Study results

Summary results
N/A