# Stop study.

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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?

| | |
|---|---|
| **Ethische beoordeling** | Positief advies |
| **Status** | Werving nog niet gestart |
| **Type aandoening** | - |
| **Onderzoekstype** | Observationeel onderzoek, zonder invasieve metingen |

## Samenvatting

### ID

NL-OMON25201

**Bron**
NTR

**Aandoening**

haemophilia, prophylaxis, adherence, bleeds, arthropathy.

hemofilie, profylaxe, therapietrouw, bloedingen, arthropathie.

### Ondersteuning

**Primaire sponsor:** University Medical Center Utrecht (UMC Utrecht)
**Overige ondersteuning:** Novo Nordisk

### Onderzoeksproduct en/of interventie

### Uitkomstmaten

**Primaire uitkomstmaten**

1. Joint status according to Haemophilia Joint Health Score (HJHS) and Pettersson score;<br>
2. Annual number of joint bleeds;<br>
3. Figure 8 walk test.

# Toelichting onderzoek

### Achtergrond van het onderzoek

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.

### Doel van het onderzoek

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?

### Onderzoeksopzet

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

### Onderzoeksproduct en/of interventie

N/A

# Contactpersonen

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## Deelname eisen

### Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

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).

### Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

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.

# Onderzoeksopzet

## Opzet

| | |
|---|---|
| Type: | Observationeel onderzoek, zonder invasieve metingen |
| Onderzoeksmodel: | Parallel |
| Toewijzing: | Niet-gerandomiseerd |
| Blindering: | Enkelblind |
| Controle: | N.v.t. / onbekend |

## Deelname

Nederland

| | |
|---|---|
| Status: | Werving nog niet gestart |
| (Verwachte) startdatum: | 01-11-2011 |
| Aantal proefpersonen: | 90 |
| Type: | Verwachte startdatum |

# Ethische beoordeling

Positief advies

| | |
|---|---|
| Datum: | 10-10-2011 |
| Soort: | Eerste indiening |

# Registraties

## Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

## Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

## In overige registers

| Register | ID |
|---|---|
| NTR-new | NL2950 |
| NTR-old | NTR3098 |
| Ander register | WHO UTN : U1111-1121-7069 |
| ISRCTN | ISRCTN wordt niet meer aangevraagd. |

# Resultaten

**Samenvatting resultaten**
N/A