Desmopressin and FVIII concentrate combination treatment in non-severe hemophilia A patients undergoing minor interventions

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Desmopressin and FVIII concentrate combination treatment will be equally effective as FVIII concentrate monotherapy, but will decrease the needed amount of FVIII concentrate significantly

Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON26618

Bron
Nationaal Trial Register

Verkorte titel
Little DAVID

Aandoening

Hemophilia A;
Surgery;
Pharmacokinetics;
Desmopressin

Ondersteuning

Primaire sponsor: Erasmus University Medical Center Rotterdam
Overige ondersteuning: Erasmus University Medical Center Rotterdam; Innovatiefonds zorgverzekeraars

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Efficacy of treatment, measured as the deviation of the measured FVIII from the predicted FVIII range preoperatively.
The amount of FVIII concentrate in IU per kilogram

Toelichting onderzoek

Achtergrond van het onderzoek

Rationale: Hemophilia A (HA) is a rare bleeding disorder, caused by factor VIII deficiency. In non-severe HA patients (FVIII 0.01-0.40 IU/mL), minor interventions, such as dental surgery and endoscopies, are an important treatment indication. Two treatment options are currently available: FVIII concentrate and desmopressin. Unfortunately, perioperative treatment is not optimal, as high bleeding rates after dental surgery, the most frequent occurring type of minor interventions, of 12% and 25% were found in two recent studies. Moreover, a previous evaluation of FVIII concentrate treatment from our centre shows a high rate of dosing above FVIII target levels (79%) and a rate of dosing below FVIII target levels of 8%. Both may lead to complications. Moreover, FVIII concentrate is expensive. Pharmacokinetic (PK) guided dosing, a patient tailored dosing method, can improve dosing accuracy.

Desmopressin, the second treatment option, releases endogenous FVIII and von Willebrand factor, improving hemostasis. Desmopressin is not ideal due to several barriers. Amongst others, most patients do not reach sufficient FVIII levels to undergo minor interventions. An increase in the use of desmopressin instead of FVIII concentrate would be highly beneficial, as desmopressin is cheaper and more widely available. Desmopressin and FVIII concentrate combination treatment may be an innovative treatment option.

Objective: To show the efficacy of combination treatment with desmopressin and FVIII concentrate is equal to the efficacy of FVIII concentrate monotherapy, but will reduce the FVIII concentrate consumption in non-severe hemophilia A patients around minor interventions.

Study design: Randomized controlled trial

Study population: Seventy non-severe hemophilia A patients (FVIII ¡Ý0.01 IU/mL), between 12-70 years of age, undergoing a minor intervention and requiring perioperative FVIII replacement therapy for a maximum of 48 hours.

Intervention (if applicable): The first group receives standard treatment consisting of FVIII concentrate monotherapy. The intervention group receives desmopressin and FVIII concentrate combination treatment.

Main study parameters/endpoints: The average deviation of the measured FVIII peak level to the predicted peak FVIII range before the minor intervention in IU per mL and the FVIII concentrate consumption in IU per kilogram.

Doel van het onderzoek

Desmopressin and FVIII concentrate combination treatment will be equally effective as FVIII concentrate monotherapy, but will decrease the needed amount of FVIII concentrate significantly

Onderzoeksopzet

preoperatively

14 days postoperatively

4-6 weeks postpoperatively

Onderzoeksproduct en/of interventie

Desmopressin and FVIII concentrate combination treatment.

Contactpersonen

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- Non-severe hemophilia A patients (FVIII 0.01-0.40 IU/mL)
- In need of a minor surgical intervention
- Age minimally 12 and maximally 70 years at study inclusion date
- Need for perioperative FVIII concentrates for a maximum of 48 hours
- Having admissible results of a desmopressin test (see paragraph 3.1)
- Absolute increase in FVIII 1 hour after desmopressin administration ≥ 0.2 IU/mL after a previous (test) dose
- Male gender
- (Parental) informed consent

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Patients with other congenital or acquired hemostatic abnormalities
- Clinically relevant FVIII inhibiting antibodies (>0.5 BU) preoperatively, unless successfully treated with immunotolerance therapy
- Needed treatment duration with FVIII concentrates longer than 48 hours
- Contraindications for desmopressin, e.g. cardiovascular disease (see appendix IV)
- Use of co-medication that has an interaction with desmopressin (see appendix IV)
- Intolerance to previous desmopressin administrations

Onderzoeksopzet

Opzet

Type: Interventie onderzoek

Onderzoeksmodel:	Parallel
Toewijzing:	Gerandomiseerd
Blindering:	Open / niet geblindeerd
Controle:	Actieve controle groep

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-11-2016
Aantal proefpersonen:	50
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Positief advies

Datum:	16-09-2016
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 47789
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL5855
NTR-old	NTR6036
CCMO	NL57682.078.16
OMON	NL-OMON47789

Resultaten