Effect of Genestein as food supplement in patients with Sanfilippo syndrome.

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Genestein inhibits the synthesis of heparan sulphate and can possibly slow down disease progression in Sanfilippo Syndrome patients.

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

Samenvatting

ID

NL-OMON21882

Bron
NTR

Verkorte titel
N/A

Aandoening

Sanfilippo syndrome (MPS III) comprises 4 closely related inborn errors of lysosomal degradation of the glycosaminoglycan heparan sulphate (GAGs). Accumulation of heparan sulfate, results in progressive and severe mental deterioration, which is the clinical hallmark of MPS III. Patients with MPS III usually die before the 3th decade. At present there is no causal therapy for patients with MPS III and treatment is symptomatic.

Ondersteuning

Primaire sponsor: AMC Amsterdam
Overige ondersteuning: Stichting Metakids
Weeshuis der Doopsgezinden Haarlem

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Does genestein significantly decrease urinary GAGs (in particular heparan sulphate) excretion in Sanfilippo patients?

Toelichting onderzoek

Achtergrond van het onderzoek

Sanfilippo syndrome (MPS III) is an inborn errors of lysosomal degradation of the glycosaminoglycan heparan sulphate (GAGs). Accumulation of heparan sulfate, results in progressive and severe mental deterioration, which is the clinical hallmark of MPS III. Genestein (an isoflavone) is a natural component of the soy-bean. Genistein inhibits GAGs synthesis in vitro and a recent small and open-label study suggested clinical relevant effects of genestein in MPS III patients.
In this study the use of genestein in MPS III patients will be compared to placebo.
The study consists of two periods of 6 months each with a wash-out period of 1 month in between. During one of the 6 months periods patients will receive Genestein as supplement (10 mg/kg). The other 6 months period patients will receive placebo.
During each study period patients will be studied for the effects of genestein on:
urinary GAG excretion, plasma heparansulfate, tissue (skin biopsy) concentration of heparansulfate, hair structure, cognitive state and behavioural abnormalities.

Doel van het onderzoek

Genestein inhibits the synthesis of heparan sulphate and can possibly slow down disease progression in Sanfilippo Syndrome patients.

Onderzoeksopzet

Baseline, 3 month, 6 months, 7 months, 10 months and 13 months and monthly urine samples.

Onderzoeksproduct en/of interventie

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Contactpersonen

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. The patient should have a biochemically confirmed deficiency of heparan-N-sulfatase (MPSIIIA), A-N-acetylglucosaminidase (MPS IIIB) or Acetyl CoA:ƒÑ-glucosaminide N-acetyltransferase (MPS IIIC);

2. Patients should be able to walk soms steps independent and make some form of contact.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. The parent or legal representative is unwilling to participate;

2. Patient is already using Genestein;

3. Patient received an cord blood transplantation.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Cross-over
Toewijzing:	Gerandomiseerd
Blindering:	Dubbelblind
Controle:	Placebo

Deelname

Nederland

Status:	Werving gestopt
(Verwachte) startdatum:	15-06-2009
Aantal proefpersonen:	30
Type:	Werkelijke startdatum

Ethische beoordeling

Positief advies

Datum:	19-05-2009
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	NL1716
NTR-old	NTR1826
Ander register	MEC AMC : 08/309
ISRCTN	ISRCTN wordt niet meer aangevraagd

Resultaten

Samenvatting resultaten
N/A