Efficacy of genestein as food supplement in patients with the Sanfilippo syndrome (an extension study).

Gepubliceerd: 05-07-2010 Laatst bijgewerkt: 07-12-2022

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-OMON22208

Bron
NTR

Verkorte titel
N/A

Aandoening

Sanfilippo syndrome (MPS III)

Ondersteuning

Primaire sponsor: Academic Medical Center Amsterdam (AMC)
Overige ondersteuning: Stichting Kinderen en Kansen

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 June 2009 a double blind placebo controlled study on the effect of genestein was initiated in the Netherlands. Thirty patients with MPS III type A, B or C are included in this study and receiving either placebo or genestein (10 mg/kg) during two 6 month periods in a cross-over design The outcome of genestein on urinary GAG excretion, plasma heparan sulphate, tissue (skin biopsy) concentration of heparan sulphate, hair structure, cognitive state and behavioural abnormalities will be studied. Currently the trial is still ongoing and first results are expected the end of 2010. Our study will be the extension study of the previous mentioned study.

Doel van het onderzoek

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

Onderzoeksopzet

Baseline, 6 months and 12 months.

Onderzoeksproduct en/of interventie

During a 12 month period all particpating patients will receive genestein as food supplement. Genestein dose will be calculated according to the weight of the patients (10 mg/kg/day). The start assessment of this study equals the last assessment of the double blind controlled trial.

Contactpersonen

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

Patients participated in the initial double blind placebo controlled study on the effect of genestein in a cross-over design.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

The parent or legal representative is unwilling to participate.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestopt
(Verwachte) startdatum:	12-07-2010
Aantal proefpersonen:	28
Type:	Werkelijke startdatum

Ethische beoordeling

Positief advies

Datum:	05-07-2010
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	NL2276
NTR-old	NTR2402
Ander register	MEC AMC : 10/080
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Resultaten

Samenvatting resultaten

N/A