An open randomised study comparing efficacy of maintenance therapy with imiglucerase at a frequency of once every four weeks versus the original schedule (once every one or two weeks) in adult type I Gaucher disease patients.

Gepubliceerd: 12-09-2006 Laatst bijgewerkt: 13-12-2022

To compare the efficacy of maintenance therapy with an equal monthly dose of imiglucerase when administered at a frequency of once every four weeks versus once every one or two weeks, in adult type I Gaucher disease patients in stable and good…

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

Samenvatting

ID

NL-OMON21048

Bron
NTR

Verkorte titel
Q2Q4

Aandoening

Type I Gaucher disease.

Ondersteuning

Primaire sponsor: N/A
Overige ondersteuning: Governmental funding of the Academic Medical Center for the centralized treatment and monitoring of Gaucher disease patients in the Netherlands.

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Stabilization of liver ratio (mL livervolume/kg bodyweight).

Toelichting onderzoek

Achtergrond van het onderzoek

Gaucher disease type I can be successfully treated with enzyme replacement therapy. In order to reduce the burden of the intravenously administered enzyme, low frequency of infusion will be prospectively studied in patients with stable and minor disease following ERT. Patients will be randomly assigned to continue their original regimen (in a once every week or fortnightly schedule) or to lower the rate of infusion to once every four weeks, at the same cumulative dose. Primary endpoint is change in liver ratio (ml/kg body weight) and secondary endpoints are spleen volume, haemoglobin level, platelet count, lumbar bone marrow fat content measured with QCSI, white cell count, and plasma levels of ferritin, chitotriosidase, liver enzymes and angiotensin converting enzyme (ACE).

Doel van het onderzoek

To compare the efficacy of maintenance therapy with an equal monthly dose of imiglucerase when administered at a frequency of once every four weeks versus once every one or two weeks, in adult type I Gaucher disease patients in stable and good condition during a minimum of two years on enzyme supplementation therapy.

Onderzoeksopzet

N/A

Onderzoeksproduct en/of interventie

Lowering of the frequency of enzyme replacement therapy to once every four weeks.

Contactpersonen

Publiek

Academic Medical Center (AMC),
Department of Internal Medicine, F4-279,
P.O. Box 22660
C.E.M. Hollak
Meibergdreef 9
Amsterdam 1100 DD
The Netherlands
+31 (0)20 5666071

Wetenschappelijk

Academic Medical Center (AMC),
Department of Internal Medicine, F4-279,
P.O. Box 22660
C.E.M. Hollak
Meibergdreef 9
Amsterdam 1100 DD
The Netherlands
+31 (0)20 5666071

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. Patients, older than 18 years, with proven Gaucher type I disease, as evidenced by decreased plasma glucocerebrosidase activity or genotyping.

2. Patients who have received enzyme therapy for at least two years prior to study enrolment..

3. Patients with mild, stable Gaucher disease, as defined by having all of the following throughout the 24 months prior to screening:

a. haemoglobin levels within normal limits (male >8.0 mmol/L, female >7.5 mmol/L)

b. platelet count >100 x 109/L

c. no or asymptomatic organomegaly

d. no evidence of clinical bone disease, such as avascular necrosis, pathologic fractures, orthopaedic replacement or bone-crises.

e. QCSI levels of > 23%

f. a maximum variability of 30% in plasma chitotriosidase levels

4. Patients who have provided written informed consent to participate in the study.

5. Patients who are co-operative, able to understand the nature and scope of the study, and who are expected to be generally compliant.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

N/A

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Toewijzing:	Gerandomiseerd
Blindering:	Open / niet geblindeerd
Controle:	Geneesmiddel

Deelname

Nederland

Status:	Werving gestopt
(Verwachte) startdatum:	28-05-2003
Aantal proefpersonen:	11
Type:	Werkelijke startdatum

Ethische beoordeling

Positief advies

Datum:	12-09-2006
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	NL724
NTR-old	NTR734
Ander register	: N/A
ISRCTN	ISRCTN51027260

Resultaten

Samenvatting resultaten
Haematologica. 2007 Feb;92(2):215-21.