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.

No registrations found.

Ethical review	Positive opinion
Status	Recruitment stopped
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON21048

Source
NTR

Brief title
Q2Q4

Health condition

Type I Gaucher disease.

Sponsors and support

Primary sponsor: N/A
Source(s) of monetary or material Support: Governmental funding of the Academic Medical Center for the centralized treatment and monitoring of Gaucher disease patients in the Netherlands.

Intervention

Outcome measures

Primary outcome

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

Secondary outcome

1. Stabilization of chitotriosidase (in patients who are not deficient for the chitotriosidase gene, 6% of population);

2. Stabilization of haemoglobin and platelet count;

3. Stabilization of hexosaminidase;

4. Stabilization of spleen volume;

5. Stabilization of QCSI;

6. Change in QOL;

7. Stabilization of ASAT, ALAT, y-GT, LDH, AF, ACE, ferritin.

Study description

Background summary

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).

Study objective

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.

Study design

N/A

Intervention

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

Contacts

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Eligibility criteria

Inclusion criteria

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.

Exclusion criteria

N/A

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)
Control:	Active

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	28-05-2003

Enrollment:	11
Type:	Actual

Ethics review

Positive opinion Date:	12-09-2006
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL724
NTR-old	NTR734
Other	: N/A
ISRCTN	ISRCTN51027260

Study results

Summary results
Haematologica. 2007 Feb;92(2):215-21.