

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

Submission date 12/10/2006	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
Registration date 12/10/2006	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 15/10/2008	Condition category Nutritional, Metabolic, Endocrine	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data
		<input type="checkbox"/> Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Protocol serial number
NTR734

Study information

Scientific Title

Acronym

Q2Q4

Study objectives

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.

Ethics approval required

Old ethics approval format

Ethics approval(s)

Received from the local medical ethics committee

Primary study design

Interventional

Study design

Randomised controlled trial

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Gaucher disease

Interventions

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

1. Imiglucerase once every four weeks
2. Imiglucerase once every one or two weeks (normal therapy)

Intervention Type

Drug

Phase

Not Specified

Drug/device/biological/vaccine name(s)

Imiglucerase

Primary outcome(s)

Stabilisation of liver ratio (mL liver volume/kg body weight)

Key secondary outcome(s)

1. Stabilisation of chitotriosidase (in patients who are not deficient for the chitotriosidase gene, 6% of population)
2. Stabilisation of haemoglobin and platelet count
3. Stabilisation of hexosaminidase
4. Stabilisation of spleen volume
5. Stabilisation of QCSI
6. Change in quality of life (QOL)
7. Stabilisation of aspartate aminotransferase (ASAT), alanine aminotransferase (ALAT), gamma-glutamyl transferase (γ -GT), lactate dehydrogenase (LDH), alkaline phosphatase (AF), angiotensin converting enzyme (ACE), ferritin

Completion date

01/11/2004

Eligibility

Key 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:
 - 3.1. Haemoglobin levels within normal limits (male more than 8.0 mmol/L, female more than 7.5 mmol/L)
 - 3.2. Platelet count more than $100 \times 10^9/L$
 - 3.3. No or asymptomatic organomegaly
 - 3.4. No evidence of clinical bone disease, such as avascular necrosis, pathologic fractures, orthopaedic replacement or bone-crisis
 - 3.5. Quantitative Chemical Shift Imaging (QCSI) levels of more than 23%
 - 3.6. 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

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Lower age limit

18 Years

Sex

All

Key exclusion criteria

Does not comply with the above inclusion criteria

Date of first enrolment

28/05/2003

Date of final enrolment

01/11/2004

Locations

Countries of recruitment

Netherlands

Study participating centre

Academic Medical Center (AMC)

Amsterdam

Netherlands

1100 DD

Sponsor information

Organisation

Academic Medical Center (AMC) (The Netherlands)

ROR

<https://ror.org/03t4gr691>

Funder(s)

Funder type

Hospital/treatment centre

Funder Name

Academic Medical Center (AMC) (The Netherlands)

Alternative Name(s)

Academic Medical Center, Centre Médical Académique, ACADEMISCH MEDISCH CENTRUM AMSTERDAM, Academic Medical Center (Amsterdam), AMC

Funding Body Type

Government organisation

Funding Body Subtype

Universities (academic only)

Location

Netherlands

Results and Publications**Individual participant data (IPD) sharing plan****IPD sharing plan summary**

Not provided at time of registration