ISRCTN51027260 https://doi.org/10.1186/ISRCTN51027260

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	Recruitment status	[ ] Prospectively registered
12/10/2006	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
12/10/2006	Completed	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
15/10/2008	Nutritional, Metabolic, Endocrine	[ ] Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr C E M Hollak

Contact details
Academic Medical Center (AMC)
Department of Internal Medicine, F4-279
P.O. Box 22660
Amsterdam
Netherlands
1100 DD
+31 (0)20 5666071
c.e.hollak@amc.uva.nl

Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
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

Study design
Randomised controlled trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Not specified

Study type(s)
Treatment

Participant information sheet

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

Secondary outcome measures
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 (y-GT), lactate dehydrogenase (LDH), alkaline phosphatase (AF), angiotensin converting fnzyme (ACE), ferritin

Overall study start date
28/05/2003

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 x 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-crises
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

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
11

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)

Sponsor details
Department of Internal Medicine
P.O. Box 22660

Amsterdam
Netherlands
1100 DD

Sponsor type
Hospital/treatment centre

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, AMC

Funding Body Type
Private sector organisation

Funding Body Subtype
Universities (academic only)

Location
Netherlands

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration