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
12/10/2006

Recruitment status
No longer recruiting

Registration date
12/10/2006

Overall study status
Completed

Last Edited
15/10/2008

Condition category
Nutritional, Metabolic, Endocrine

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[ ] Results
[ ] Individual participant data
[ ] 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 (y-GT), lactate dehydrogenase (LDH), alkaline phosphatase (AF), angiotensin converting fnzyme (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 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

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