

# 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

<b>Submission date</b> 12/10/2006	<b>Recruitment status</b> No longer recruiting	<input type="checkbox"/> Prospectively registered
<b>Registration date</b> 12/10/2006	<b>Overall study status</b> Completed	<input type="checkbox"/> Protocol
<b>Last Edited</b> 15/10/2008	<b>Condition category</b> 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

## Study design

Randomised controlled trial

## Primary study design

Interventional

## 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 ( $\gamma$ -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, AMC

**Funding Body Type**

Private sector 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