Whole-body MRI in Gaucher disease; monitoring disease activity, early detection of complications and treatment decision-making

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Metabolism disorders NEC
Study type	Observational invasive

Summary

ID

NL-OMON44801

Source
ToetsingOnline

Brief title
IMAGO study

Condition

- Metabolism disorders NEC

Synonym
Gaucher disease, glucosylceramidosis

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum

Source(s) of monetary or material Support: Fonds NutsOhra

Intervention

Keyword: Gaucher disease, Ironlevel, Magnetic Resonance Imaging

Outcome measures

Primary outcome

The main study parameter is the difference in the measured ironlevels

(expressed as R2*, a MRI based value correlating with ironconcentration (µmol/

g)) between Gaucherpatients and healthy controls.

Secondary outcome

- Comparison of ironmeasurements with standard care monitoring, that is liver-

and spleenvolumes, QCSI and BMB-scores.

- Relation ironmeasurements with biochemical GD markers

- The value of extending the QCSI measurements, including tibia and fibula

- Reproducibility of the whole-body MRI protocol

Study description

Background summary

Gaucher disease (GD) is a rare lysosomal storage disorder in which a deficiency of the enzyme glucocerebrosidase leads to accumulation of its substrate glucosylceramide. This substrate accumulates in macrophages and these accumulated cells are mainly localized in spleen, liver and bone marrow, causing a range of symptoms1,2. At present, monitoring of GD is performed with the use of biochemical parameters and imaging of liver, spleen and bone marrow density using magnetic resonance imaging (MRI). There is no method available to assess the amount of accumulated cells in the entire body. A new approach in this field is imaging of the amount of iron using whole body magnetic resonance imaging (MRI). It is known that iron is an often seen component in Gauchercells 3-8 and therefore it is possible that iron could be used as a marker of Gauchercells. Using a whole body MRI iron protocol it can be possible to gain

an improved insight in severity and extent of Gaucher disease.
We hypothesize that in patients with GD the whole body MRI iron measurements are a useful tool in determining the distribution and total amount of Gauchercells in the body and can be of use in monitoring disease activity, early detection of complications and treatment decision-making.

Study objective

The objective of the study is to investigate the applicability of the whole body MRI protocol in Gaucher disease. To do so, we will investigate:
1. If whole-body MRI ironmeasurements represent the distribution and total amoount of Gauchercells in the entire body.
2. The relation of these ironmeasurements with present day monitoring parameters of Gaucher disease - biochemical and imaging.
3. If the ironmeasurements are of use in early detection of complications and treatment decision-making in Gaucher disease.
4. If the distribution and severity of residual disease is related to the risk of developing complications.

Study design

Case-control study

Study burden and risks

All participants undergo MRI-scanning and venous blood sampling at baseline, a subgroup of the participants will undergo a second MRI scan. Present day, MRI-scanning is already part of the follow-up of Gaucher patients. For this study the current scanning technique needs to be extended for 30 minutes, total scanning time is 90 minutes. Risks associated with the scanning and venous blood sampling are negligible. The potential value of this research could be the implementation of this new imaging technique in the monitoring protocol of GD, to get a better insight in disease activity and early detection of complications. It may also result in acquiring better knowledge concerning treatment decision-making. This will be of direct benefit for the subjects involved, as well as other Gaucher patients.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Age >18 years
Ability to provide informed consent
Patients: confirmed diagnosis Gaucher disease
Controls: resemblance in age and sex to matched patient

Exclusion criteria

Age <18 years
Contra-indication MRI
Controls:
- known disease/abnormalities of liver, spleen or bone marrow. Or any of these in medical history.
- possible causes of iron overload

Study design

Design

Study type:	Observational invasive
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	19-05-2014
Enrollment:	80
Type:	Actual

Ethics review

Approved WMO Date:	13-12-2013
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	11-01-2017
Application type:	Amendment
Review commission:	METC Amsterdam UMC

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
CCMO	NL46217.018.13