

Relation between enzyme replacement therapy and progression of brain lesions in Fabry disease

Submission date 27/01/2015	Recruitment status No longer recruiting	<input checked="" type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
Registration date 10/02/2015	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan <input checked="" type="checkbox"/> Results
Last Edited 21/06/2019	Condition category Nutritional, Metabolic, Endocrine	<input type="checkbox"/> Individual participant data

Plain English summary of protocol

Background and study aims

Fabry disease is an inherited disorder that results from the build-up of a particular type of fat (globotriaosylceramide) in the body's cells. Beginning in childhood, this build-up causes signs and symptoms that affect many parts of the body including the brain. In the brain, small groups of dead cells clump together in the white matter and are known as white-matter lesions. These lesions may lead to a high risk of early dementia, stroke or death. Little is known about the development of white-matter lesions and how they relate to other factors (e.g., age, sex or smoking) or how they are affected by treatment of Fabry disease with ERT. Salford Royal NHS Foundation Trust (UK) has a database of patients with Fabry disease in the northwest of England. The aim in this study is to look in detail at the relation between ERT and progression of brain lesions in Fabry disease so as to understand how the incidence and burden of the lesions change over time.

Who can participate?

Adults with Fabry disease who have had two MRI scans, 1 year apart

What does the study involve?

The size of the white-matter lesions will be measured over time and this information will be used alongside details of age, sex, risk factors for stroke/heart disease and treatment with ERT.

What are the possible benefits and risks of participating?

There are no known benefits or risks to participants taking part in this study

Where is the study run from?

Salford Royal NHS Foundation Trust (UK)

When is the study starting and how long is it expected to run for?

February 2015 to December 2015

Who is funding the study?

Investigator initiated and funded (UK)

Who is the main contact?
Mrs Sharon Hulme
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Contact information

Type(s)
Public

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Additional identifiers

ClinicalTrials.gov (NCT)
NCT00196742

Study information

Scientific Title
Magnetic resonance imaging of effect of enzyme replacement therapy on progression of cerebral white-matter lesions in Fabry disease: an observational study

Study objectives

1. Does treatment of Fabry disease with enzyme replacement therapy (ERT) affect the build up and progression of white matter lesions?
2. White matter lesions can increase the risk of stroke and dementia and it is important to assess if treatment with ERT increases this risk

Ethics approval required

Old ethics approval format

Ethics approval(s)

NRES Committee West Midlands - South Birmingham, 19/02/2015, ref: 15/WM/0064

Study design

Observational study

Primary study design

Observational

Study type(s)

Other

Health condition(s) or problem(s) studied

Fabry disease

Interventions

Retrospective analysis of a database and serial magnetic resonance imaging (MRI) scans to assess:

1. Progression of white matter lesions in patients with Fabry disease
2. Relation between disease progression and treatment with ERT

Intervention Type

Biological/Vaccine

Phase

Not Applicable

Primary outcome(s)

Prevalence and burden of white matter lesions over time: MRI scans at baseline and at 2 years will be compared for evidence of white-matter lesions using a visual severity rating scale

Key secondary outcome(s)

Progression of white matter lesions: MRI scans at baseline and at 2 years will be compared for evidence of white-matter lesions using a visual severity rating scale

Completion date

31/12/2016

Eligibility

Key inclusion criteria

1. Confirmed diagnosis of Fabry disease
2. Age at least 18 years old
3. Being followed up at Salford Royal NHS Foundation Trust (UK)
4. Registered in Fabry disease registry
5. At least two serial MRI brain scans (1 year apart)

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Lower age limit

18 years

Sex

All

Total final enrolment

149

Key exclusion criteria

1. New patient
2. No serial MRI scans
3. MRI scans of insufficient quality for analysis

Date of first enrolment

15/02/2015

Date of final enrolment

31/12/2015

Locations**Countries of recruitment**

United Kingdom

England

Study participating centre

Salford Royal NHS Foundation Trust

Clinical Sciences Building

Stott lane

Salford

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M6 8HD

Sponsor information

Organisation

University of Manchester

ROR

<https://ror.org/027m9bs27>

Funder(s)

Funder type

Not defined

Funder Name

Investigator initiated and funded (UK)

Results and Publications

Individual participant data (IPD) sharing plan

The datasets generated during and/or analysed during the current study are/will be available upon request from Dr Ana Jovanovic Ana.Jovanovic@srft.nhs.uk

IPD sharing plan summary

Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	09/10/2018	14/06/2019	Yes	No
HRA research summary			28/06/2023	No	No