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Treatment of Fabry patients greater than 18 years with enzyme supplementation therapy: comparison of efficacy and toxicity of low dose (0.2 mg/kg) Fabrazyme® (agalsidase beta) or Replagal® (agalsidase alfa)

Submission date
20/12/2005

Recruitment status
No longer recruiting

Registration date
20/12/2005

Overall study status
Completed

Last Edited
04/07/2019

Condition category
Nutritional, Metabolic, Endocrine

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

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
NTR216

Study information

Scientific Title
Treatment of Fabry patients greater than 18 years with enzyme supplementation therapy: comparison of efficacy and toxicity of low dose (0.2 mg/kg) Fabrazyme® (agalsidase beta) or Replagal® (agalsidase alfa)

Study objectives
Evaluation of efficacy and safety of two different formulas of alfa-Galactosidase A, agalsidase beta (Fabrazyme®) and agalsidase alpha (Replagal®) in an equal dose of 0.2 mg/kg in order to detect any differences between these two drugs.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Received from the local medical ethics committee

Primary study design
Interventional

Study design
Multicentre, randomised, active controlled, factorial trial

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Fabry disease

Interventions
Patients will receive 0.2 mg/kg Fabrazyme® (agalsidase beta) or 0.2 mg/kg Replagal®(agalsidase alpha), every two weeks for a minumum of 12 months. If there is treatment failure (progression of renal disease, cardiac disease and/or a new cerebral stroke or TIA) during or after this period, patients will be advised to switch to Fabrazyme 1.0 mg/kg/2 weeks.

Intervention Type
Drug

Phase
Not Specified

Drug/device/biological/vaccine name(s)
Agalsidase beta (Fabrazyme®), agalsidase alpha (Replagal®)

Primary outcome(s)
Wall-thickness (septum and left and right ventricle wall)/end-diastolic volume) on echocardiography.

Key secondary outcome(s)

1. Improvement of renal function as measured by GFR
2. Reduction of glycolipid accumulation in skin tissue (LM and biochemistry)
3. Reduction in pain as measured by the BPI
4. Reduction in glycosphingolipid in plasma and 24-hr urine
5. Quality of life scores (36-item Short Form Health Survey [SF-36])

Completion date
31/12/2005

Eligibility

Key inclusion criteria
1. The patient must have given written informed consent
2. Patients must be 18 years or older
3. Patient must have a current diagnosis of Fabry disease
4. Patients must have a decreased alpha-Gal activity or proven alfa-Gal A mutation
5. Female patients must have a negative pregnancy test, and must use a medically accepted method of contraception
6. Patients must be willing to comply to the evaluation program
7. Patients must have a clinical presentation consistent with either typical or atypical Fabry disease

Patients must have at least one major or two minor objective criteria:
Major:
1. Severe acroparesthesias, that cannot satisfactorily be controlled with Carbamazepine
2. Decreased glomerular filtration rate (GFR) less than 80 ml/min
3. Proteinuria greater than 300 mg/ml
4. Documented cerebrovascular accident (CVA)
5. Cardiac infarction
6. Hypertrophic non-obstructive cardiomyopathy resulting in decreased exercise tolerance
7. Rhythm disturbances necessitating a pacemaker
8. Multiple lacunar infarctions on magnetic resonance imaging (MRI)

Minor:
1. Documented transient ischaemic attack (TIA)
2. Cardiac hypertrophy on echo or MRI
3. Atrial fibrillation
4. Intraventricular conduction abnormality
5. Sensoric hearing loss as shown on a hearing test
6. Severe vertigo
7. Micro-albuminuria greater than 50 mg/L
8. Mild to moderate acroparesthesias
9. Gastro-intestinal complaints that can not be explained by other medical conditions than Fabry disease

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 Years

Sex
All

Total final enrolment
34

Key exclusion criteria
1. Patient is pregnant or lactating
2. Patient is unwilling to comply to the evaluation program

Date of first enrolment
29/05/2001

Date of final enrolment
31/12/2005

Locations

Countries of recruitment
Netherlands

Study participating centre
Academic Medical Centre
Amsterdam
Netherlands
1105 AZ

Sponsor information

Organisation
Academic Medical Centre (AMC) (The Netherlands)

ROR
https://ror.org/03t4gr691

Funder(s)

Funder type
Government

Funder Name
The Dutch Health Care Insurance Board (CVZ) (The Netherlands)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	11/07/2007	04/07/2019	Yes	No