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Protocolised follow-up of Pompe patients receiving enzyme replacement therapy on a compassionate use basis

Submission date	Recruitment status	[ ] Prospectively registered
23/02/2007	Recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
23/02/2007	Ongoing	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
01/07/2016	Nutritional, Metabolic, Endocrine	[ ] 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

Study information

Scientific Title
Protocolised follow-up of Pompe patients receiving enzyme replacement therapy on a compassionate use basis

Study objectives
Enzyme therapy with recombinant human alpha glucosidase results in:
1. Prolonged survival
2. Improvement or stabilisation of cardiac hypertrophy and function
3. Improvement or stabilisation of pulmonary function
4. Improvement or stabilisation of muscle function and strength

Ethics approval required
Old ethics approval format

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

Study design
Protocolised follow up of parallel group trial

Primary study design
Observational

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Pompe Disease

Interventions
Enzyme replacement therapy

Intervention Type
Drug

Phase
Not Specified

Drug/device/biological/vaccine name(s)
Recombinant human alpha glucosidase

Primary outcome(s)
1. Infantile: Survival
2. Late-onset: Improvement and/or stabilisation of muscle function

Key secondary outcome(s))
1. Infantile:
a. improvement of cardiac hypertrophy and function
b. achievement of motor milestones
2. Late-onset:
a. improvement and/or stabilisation of pulmonary function
b. improvement of quality of life

Completion date

01/01/2050

Eligibility

Key inclusion criteria
1. Confirmed diagnosis of Pompe Disease
2. Infantile-onset:
2.1. Age less than one year
2.2. Delayed motor milestones, and/or
2.3. Hypertrophic cardiomyopathy
3. Late-onset:
3.1. 24 hour/day artificial ventilation
3.2. Wheelchair bound
3.3. Previously enrolled in AGLU 1202 study

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Not Specified

Sex
Not Specified

Key exclusion criteria
1. Infantile-onset:
1.1. congenital abnormalities
1.2. allergy to food and/or proteins
1.3. ventilator dependency
2. Late-onset:
2.1. developmental delays not explained by Pompe's Disease
2.2. allergies
2.3. severe co-morbidity

Date of first enrolment
01/01/1999

Date of final enrolment
01/01/2050

Locations

Countries of recruitment
Netherlands

Study participating centre
Erasmus Medical Centre
Rotterdam
Netherlands
3000 CB

Sponsor information

Organisation
Erasmus Medical Centre (Netherlands)

ROR
https://ror.org/018906e22

Funder(s)

Funder type
Industry

Funder Name
Genzyme Corporation (Netherlands)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration