

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

Submission date 23/02/2007	Recruitment status Recruiting	<input type="checkbox"/> Prospectively registered
		<input type="checkbox"/> Protocol
Registration date 23/02/2007	Overall study status Ongoing	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
Last Edited 01/07/2016	Condition category Nutritional, Metabolic, Endocrine	<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

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers

N/A

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

Secondary study design

Single-centre

Study setting(s)

Other

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use contact details to request a participant information sheet

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 measure

1. Infantile: Survival
2. Late-onset: Improvement and/or stabilisation of muscle function

Secondary outcome measures

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

Overall study start date

01/01/1999

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

Age group

Not Specified

Sex

Not Specified

Target number of participants

12

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)

Sponsor details

Sophia Children's Hospital

Dr. Molewaterplein 60

Rotterdam

Netherlands

3015 GJ

Sponsor type

Hospital/treatment centre

Website

<http://www.erasmusmc.nl/>

ROR

<https://ror.org/018906e22>

Funder(s)

Funder type

Industry

Funder Name

Genzyme Corporation (Netherlands)

Results and Publications

Publication and dissemination plan

Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration