ISRCTN72578000 https://doi.org/10.1186/ISRCTN72578000

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

Submission date	Recruitment status
23/02/2007	Recruiting
Registration date	**Overall study status**
23/02/2007	Ongoing
Last Edited	**Condition category**
01/07/2016	Nutritional, Metabolic, Endocrine

- [ ] Prospectively registered
- [ ] Protocol
- [ ] Statistical analysis plan
- [ ] Results
- [ ] Individual participant data
- [ ] Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr A van der Ploeg

Contact details
Erasmus Medical Centre
Sophia Children's Hospital
PO Box 2060
Rotterdam
Netherlands
3000 CB
+31 (0)10 463 7044
a.vanderploeg@erasmusmc.nl

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