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

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON21598

Source
NTR

Brief title
N/A

Health condition

Pompe Disease

Sponsors and support

Primary sponsor: Investigator initiated. Enzyme supplied by Genzyme Corporation.
Source(s) of monetary or material Support: None.

Intervention

Outcome measures

Primary outcome

Infantile: Survival;

Late-onset: Improvement and/or stabilisation of muscle function.

Secondary outcome

Infantile: improvement of cardiac hypertrophy and function, achievement of motor milestones;

Late-onset: improvement and/or stabilisation of pulmonary function, improvement of quality of life.

Study description

Background summary

Protocollised follow-up of cardio-pulmonary function and musculo-skeletal functio in Pompe patients receiving enzyme replacement therapy on a compessionate use basis.

Study objective

Enzyme therapy with recombinant human alpha glucosidase results in prolonged survival; improvement or stabilisation of cardiac hypertrophy and function, improvement or stabilisation of pulmonary function and improvement or stabilisation of muscle function and strength.

Intervention

Enzyme replacement therapy.

Contacts

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Eligibility criteria

Inclusion criteria

Confirmed diagnosis of Pompe Disease
infantile-onset: age less than 1 year, delayed motor milestones and/or hypertrophic cardiomyopathy.

late-onset 1.: 24 hour/day artificial ventilation, wheelchair bound or previously enrolled in AGLU 1202 study.

Exclusion criteria

Infantile-onset: congenital abnormalities, allergy to food and/or proteins, ventilator dependency;

Late-onset: developmental delays not explained by Pompe's Disease, allergies and severe co-morbidity.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Masking:	Open (masking not used)
Control:	Active

Recruitment

NL
Recruitment status: Recruiting

Start date (anticipated):	01-01-1999
Enrollment:	12
Type:	Anticipated

Ethics review

Positive opinion Date:	12-09-2005
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL334
NTR-old	NTR372
Other	: N/A
ISRCTN	ISRCTN72578000

Study results

Summary results
Recombinant human alpha-glucosidase from rabbit milk in Pompa patients (The Lancet 2000).
Enzyme therapy for pompe's disease with recombinant human alpha-glucosidase from rabbit milk (J. Inherit. Metab. Dis. 2001)

Long term IV treatment of pompe's disease with recombinant human alpha-glucosidase from milk (pediatrics 2004)
Enzyme replacement therapy in late-onset Pompe's disease: a three year follow-up (Ann. Neurology 2004)
Morphological changes in muscle tissue of patients with Infantile Pompe's disease receiving enzym replacement therapy (Muscle Nerv 2003).