An Open-Label Extension Study of Patients with Late-Onset Pompe Disease Who Were Previously Enrolled in Protocol AGLU02704

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The objective of this extension study is to assess the long-term safety and efficacy of Myozyme treatment in patients with Late-Onset Pompe Disease who were previously treated under the placebo-controlled, double-blind study AGLU02704.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Inborn errors of metabolism
Study type	Interventional

Summary

ID

NL-OMON30570

Source
ToetsingOnline

Brief title
Open-label extension study to LOTS

Condition

- Inborn errors of metabolism
- Muscle disorders
- Neuromuscular disorders

Synonym
late-onset Pompe disease, Pompe's disease

Research involving
Human

Sponsors and support

Primary sponsor: Genzyme
Source(s) of monetary or material Support: Genzyme

Intervention

Keyword: enzyme replacement therapy (ERT) open-label, Myozyme, Pompe disease (Late-Onset)

Outcome measures

Primary outcome

1. Change from Treatment Baseline to End of Study time point in 6MWT - meters

walked

2. Change from Treatment Baseline to End of Study time point in % predicted FVC

in the upright position

Secondary outcome

1. Change from Treatment Baseline to End of Study time point in QMT Leg Score

for % predicted bilateral knee flexors and knee extensors

2. Change from Treatment Baseline to End of Study time point in PCS score of

the MOS SF-36 Health Survey

Study description

Background summary

The patients are currently particpating in a placebo controlled study (AGLU02704) which has recently been extended to 78 weeks of treatment. After completion of this study, patients will be asked to take part in an open-label extension study (AGLU03206).

Study objective

The objective of this extension study is to assess the long-term safety and

efficacy of Myozyme treatment in patients with Late-Onset Pompe Disease who were previously treated under the placebo-controlled, double-blind study AGLU02704.

Study design

Open-label treatment with Myozyme.

Intervention

Intravenous infusion with Myozyme (20 mg/kg) qow.

Study burden and risks

Depending on of the type of visit: Day 0 one day; Week 12, Week 26 two days; all other visits last approximately one day.

During the study the following non-invasive assessments will be conducted: questionnaires (SF-36 Health survey, Fatigue Severity Scale, Rotterdam 9-Item handicap Scale); ventilator use diary (only for patients who use a ventilator); pulmonary function testing; quantitative and manual muscle testing; functional activities testing; 6 minutes walk test; physical examination; height and weight; blood pressure, pulse, respiratory rate and temperature; hearing test; 12-lead ECG; urinalysis; echo; telephone contact.

During the study the following invasive assessments will be conducted: intravenous canula (IV injection) will be used for infusion of Myozyme every two weeks for a total of 14 times. The infusion volume is dependent on the weight of the patient. The intravenous canula can be used for blood sample collection to assess chemistry and hematology (7 ml each time) and anti-Myozyme antibodies (IgG: 10 ml each time). During the whole study approximately 68 ml of blood will be drawn. This amount does not include any blood samples drawn because of medical necessity.

Recently Myozyme received regulatory approval in Europe (29th of March 2006) and in the United States (28th of April 2006) for the long-term treatment of patients with Pompe disease. However, the authorities in Europe (EMEA) and the United States of America (FDA) are requiring Genzyme to complete this extended study to expand the current knowledge on Myozyme and its effectiveness and safety for treating Late Onset Pompe patients. With the successful completion of this study we hope to collect more data on the long-term effects of Myozyme which support the efficacy and safety in patients with Late Onset Pompe disease. This information will be submitted to the regulatory authorities in Europe and the United States of America. Based upon the results in patients with the infantile-onset of Pompe disease, an improvement in pulmonary function and muscle strenght can be expected in patients with Late Onset Pompe disease.

We anticipate that the results of the AGLU02704 study will confirm this hypothesis.

Treatment with Myozyme can cause side-effects. Up to now, infusions with Myozyme have been well tolerated and the most commonly reported side-effects on the day of the infusion were hypersensitivityreactions with the following symptoms: headache, redness in the face, fever, rash, urticaria, change in blood pressure and increased heart rate.

Other side-effects which are currently unknown can occur. The treatment could include risks which can currently not be predicted.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1. The patient must have completed Protocol AGLU02704, *A Randomized, Double-Blind, Multicenter, Multinational, Placebo-Controlled Study of the Safety, Efficacy, and Pharmacokinetics of Myozyme, Recombinant Human Acid alpha-Glucosidase (rhGAA), Treatment in Patients with Late-Onset Pompe Disease*, OR for patients who reside in a region where Myozyme is not available through government reimbursement or charitable access mechanisms, the patient must have completed a minimum of 52 weeks in Protocol AGLU02704;
2. The patient must provide signed, informed consent prior to performing any study-related procedures. Consent of a legally authorized guardian(s) is (are) required for patients under 18 years of age. If the patient is under 18 years of age and can understand the written informed consent, signature will be required from both the patient and the authorized guardian(s);
3. The patient (and patient*s legal guardian if patient is under 18 years of age) must have the ability to comply with the clinical protocol;
4. A female patient of childbearing potential must have a negative pregnancy test (urine beta-human chorionic gonadotropin [β-hCG]) at Baseline. Note: All female patients of childbearing potential and sexually mature males must use a medically accepted method of contraception throughout the study.

Exclusion criteria

1. The patient has a medical condition, serious intercurrent illness, or other extenuating circumstance that, in the opinion of the Investigator, would preclude treatment with Myozyme.

Study design

Design

Study phase:	4
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	02-05-2008
Enrollment:	21
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Myozyme
Generic name:	recombinant human acid-alpha glucosidase (rhGAA)
Registration:	Yes - NL intended use

Ethics review

Approved WMO Date:	09-07-2007
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	01-08-2007
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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
EudraCT	EUCTR2006-003644-31-NL
CCMO	NL15351.078.07