An Abdominal Imaging, Substrate Analysis and Laboratory Sample Collection sub-study for Participants Who Have enrolled in LAL-2-NH01

Published: 12-12-2012
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The aim of this study is to develop a better understanding of the clinical phenotype of LAL Deficiency/CESD phenotype to support the design and interpretation of planned clinical studies with SBC-102 and to inform and enhance the evaluation and care…

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Metabolic and nutritional disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON37869

Source
ToetsingOnline

Brief title
LAL-2-NH01

Condition

- Metabolic and nutritional disorders congenital

Synonym
Lipid storage disease, Wolman Disease

Research involving
Human

Sponsors and support

Primary sponsor: Synageva Biopharma Corp

Source(s) of monetary or material Support: Synageva Biopharma

Intervention

Keyword: Cholesteryl Ester Storage Disease, LAL-deficiency

Outcome measures

Primary outcome

This is an observational study, no endpoints as such are defined.

Secondary outcome

N A

Study description

Background summary

Lysosomal Acid Lipase (LAL) Deficiency is a very rare lysosomal storage disease (LSD) characterized by a failure to break down cholesteryl esters and triglycerides in lysosomes due to a deficiency of the enzyme. LAL Deficiency is a multi-system disease that most commonly manifests with gastrointestinal, liver, and cardiovascular complications and is associated with significant morbidity and mortality.

At present there are no approved therapies to treat patients with LAL Deficiency.

Synageva BioPharma Corp. is developing SBC-102, an enzyme replacement therapy, for the treatment of LAL Deficiency.

Study objective

The aim of this study is to develop a better understanding of the clinical phenotype of LAL Deficiency/CESD phenotype to support the design and interpretation of planned clinical studies with SBC-102 and to inform and enhance the evaluation and care of patients with this disease.

Study design

The substudy is a non-interventional study.

In the substudy, 2 MRI scans will be taken, as well as 3 blood samples taken at different time points.

Study burden and risks

As no treatment will be given, there is no risk involved in participating in this study.

In the substudy the patient will undergo 2 MRI scans, as well as 3 blood draws (at different time points).

All data that are collected during this study may also contribute to a better understanding of the patient's disease and may improve treatment.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- 8 years or older (only adult patients will be included in NL)
- No medical condition that would prevent from undergoing MRI or MRS
- Ability to understand the full nature and purpose of the study
- Confirmation of LAL deficiency

Exclusion criteria

- Contraindications for MRI scanning

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	08-01-2013
Enrollment:	6
Type:	Actual

Ethics review

Approved WMO

Date:	12-12-2012
Application type:	First submission
Review commission:	METC Amsterdam UMC

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
CCMO	NL40229.018.12

Study results

Date completed: 10-01-2013

Actual enrolment: 2

Summary results
Trial is onging in other countries