Bezafibrate in X-linked adrenoleukodystrophy (X-ALD).

No registrations found.

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

Summary

ID

NL-OMON24772

Source
NTR

Brief title
BEZA

Health condition

X-linked adrenoleukodystrophy (X-ALD), AMN phenotype.

Sponsors and support

Primary sponsor: University of Amsterdam, Academic Medical Center, Department of Neurology and Department of Genetic Metabolic Diseases.
Source(s) of monetary or material Support: Stop ALD

Intervention

Outcome measures

Primary outcome

1. Cholesterol (total-, LDL-, and HDL-cholesterol) and triglycerides in plasma;

2. VLCFA (C22:0, C24:0 and C26:0) in plasma, leukocytes and erythrocytes;

3. C26:0-lysophosphatidylcholine in blood.

Secondary outcome

Side effects reported by patients and abnormalities in the safety lab.

Study description

Background summary

Background of the study:

X-linked adrenoleukodystrophy (X-ALD) is an inherited metabolic disorder characterised by accumulation of very long chain fatty acids (VLCFA) in plasma and tissue. Presumably this accumulation is responsible for tissue damage. The disease can cause severe demyelinisation of the central nervous system usually causing death in childhood or progressive ambulatory problems in adults caused by a progressive myelopathy. For the latter category of patients no curative treatment is currently available. Recent investigations in human fibroblasts and mice identified bezafibrate as an agent that might reduce VLCFA in patients with X-ALD.

Objective of the study:

The trial is designed as an open-label pilot study. The main goal is to investigate if bezafibrate can reduce VLCFA in vivo in patient with X-ALD. If there is indeed a biochemical effect, a large follow-up study will be initiated with clinical outcome parameters.

Study design:

10 men with X-ALD will use bezafibrate during a period of 6 months (in combination with a low fat diet). On 6 different time points the participants will undergo a vanapuncture for detecting possible side effects and to determine the biochemical outcome parameters.

Study population:

Adult men with X-linked adrenoleukodystrophy.

Intervention:

Bezafibrate.

Primary study parameters/outcome of the study:

The primary outcome parameters are cholesterol levels (total-, LDL, and HDL) and triglycerid levels in plasma, VLCFA levels in plasma, leukocytes and erythrocytes and also C26:0-lyso-PC in bloodspots.

Secondary study parameters/outcome of the study:

Secondary outcome parameters are side-effects (subjective and abnormalities in the safety lab).

Study objective

The primary objective of this study is to determine if bezafibrate can reduce VLCFA in plasma, lymphocytes and erythrocytes and C26:0-lysophosphatidylcholine in patients with X-linked adrenoleukodystrophy.

Study design

0, 4, 8, 12, 16, 20 and 24 weeks.

Intervention

Bezafibrate 400 mg 1 pd during three months, followed by bezafibrate 400 mg 2 pd during three months.

Contacts

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Eligibility criteria

Inclusion criteria

1. An age of 18 years or older;

2. Capable of giving informed consent and physically capable of visiting the hospital for follow-up visits;

3. No contra-indications for the use of bezafibrate, e.g. kidney- and/or liver disease;

4. Confirmed X-ALD, AMN phenotype (confirmed by VLCFA analysis and mutation analysis of the ABCD1 gene).

Exclusion criteria

1. Use of medication that lowers cholesterol and/or triglycerides (e.g. statins);

2. Liver disease or and increase in serum CK of more than 3 times the baseline level;

3. Treatment with Lorenzo’s oil in the 8 weeks preceding the trial.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Non controlled trial
Masking:	Open (masking not used)

Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	06-07-2010
Enrollment:	10
Type:	Anticipated

Ethics review

Positive opinion

Date:	10-07-2010
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	NL2284
NTR-old	NTR2411
Other	MEC AMC : 09/278
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Study results

Summary results
N/A