Effect of bezafibrate on very long chain fatty acid (VLCFA) metabolism in men with X-linked adrenoleukodystrophy.

Published: 18-06-2010
Last updated: 04-05-2024

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Ethical review	Approved WMO
Status	Pending
Health condition type	Metabolic and nutritional disorders congenital
Study type	Interventional

Summary

ID

NL-OMON32731

Source
ToetsingOnline

Brief title
BEZA

Condition

- Metabolic and nutritional disorders congenital
- Spinal cord and nerve root disorders

Synonym
Schilder's disease, X-linked adrenoleukodystrofie

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: bezafibrate VLCFA X-ALD

Outcome measures

Primary outcome

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 outcome

Secondary outcome parameters are side-effects (subjective and abnormalities in

the safety lab).

Study description

Background summary

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.

Study objective

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

12 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.

Intervention

Bezafibrate.

Study burden and risks

In rare cases bezafibrate can cause a (reversible) increase in liver transaminases and/or rhabdomyolysis. This is a rare side effect. Patients will also be required to visit the hospital 6 times for a venapuncture and will be on study medication (bezafibrate) for a period of 6 months.

Contacts

Public
Academisch Medisch Centrum

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)

Elderly (65 years and older)

Inclusion criteria

- 18 years of age or older
- mentally competent and able to give informed consent and able to visit the hospital for follow-up
- no contra-indications for the use of the trial medication (kidney- and/or liver failure)
- confirmed diagnosis of X-ALD (by ABCD1 mutation analysis or abnormal plasma VLCFA)

Exclusion criteria

- Use of other medication that influences plasma cholesterol and/or triglycerid levels (for instance drugs of the statin class)
- Liver disease or increased CK (> 3 times upper limit of normal)
- Treament with VLCFA lowering agent (i.e. Lorenzo's oil).
- Unable to give informed consent

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-01-2010
Enrollment:	8
Type:	Anticipated

Medical products/devices used

Product type:	Medicine

Brand name:	Bezalip
Generic name:	bezafibrate
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO 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
EudraCT	EUCTR2009-015732-15-NL
CCMO	NL30249.018.09