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Efficacy and safety of ezetimibe in young children with familial hypercholesterolemia

Submission date	Recruitment status	[X] Prospectively registered
07/06/2006	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
07/06/2006	Completed	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
07/06/2006	Nutritional, Metabolic, Endocrine	[ ] Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr M.D. Trip

Contact details
Academic Medical Center (AMC)
Department of Cardiology
F4- 109
P.O. Box 22660
Amsterdam
Netherlands
1100 DD
+31 (0)20 5665882 or 020-5669111
m.d.trip@amc.uva.nl

Additional identifiers

Protocol serial number
N/A

Study information

Scientific Title

Acronym
EZKIMO

Study objectives
Ezetimibe monotherapy lowers low density lipoprotein-cholesterol (LDL-C) levels, plant sterol levels and inflammatory markers in young children with familial hypercholesterolemia (FH).

Ethics approval required
Old ethics approval format

Ethics approval(s)
Not provided at time of registration

Study design
Randomized, placebo-controlled trial

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Familial hypercholesterolemia (FH)

Interventions
Ezetimibe 10 mg/day versus placebo treatment for 4 months

Intervention Type
Drug

Phase
Not Specified

Drug/device/biological/vaccine name(s)
Ezetimibe

Primary outcome(s)
Primary endpoint will be the efficacy towards LDL-C levels and the safety of 10 mg ezetimibe.

Key secondary outcome(s))
Secondary endpoint will be the effect of 10 mg ezetimibe on inflammatory markers and plant sterols in plasma.

Completion date
01/08/2007

Eligibility

Key inclusion criteria

1. Male or female
2. Aged 8-14 years
3. Heterozygous familial hypercholesterolemia defined as:
a. Molecular diagnosis of FH AND LDL-C above 95th percentile for age and sex (LDL-C >3.88 mmol/l) despite a lipid-lowering diet for at least 3 months
b. LDL-cholesterol above 95th percentile for age and sex (LDL-C >3.88 mmol/l) despite a lipid-lowering diet for at least 3 months
c. One parent with either a clinical or molecular diagnosis of FH

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Child

Lower age limit
8 years

Upper age limit
14 years

Sex
All

Key exclusion criteria
1. Homozygous familial hypercholesterolemia
2. Diseases that cause a secondary increase in LDL-C, such as diabetes mellitus, anorexia nervosa and renal, hepatic or thyroid disease
3. Length below the 3rd percentile for age and sex
4. Weight-compared-to-length above the 97th percentile for age and sex
5. Serious illness in the previous three months
6. Major surgery in the previous three months
7. Partial ileal bypass or any gastrointestinal disease that might interfere with drug absorption
8. Plasma triglycerides above 4.0 mmol/l
9. Hypertension (systolic >160 mmHg or diastolic >100 mmHg)
10. Psychological disorders that might interfere with adherence to the protocol
11. Pregnancy at baseline
12. History of allergy or sensitivity to ezetimibe
13. Liver function tests, aspartate aminotransferase or alanine aminotransferase (ASAT or ALAT), must be <1.5 times the upper limit of normal (ULN) using the central laboratory reference range
14. Creatinine clearance levels must be <1.5 times the ULN using the central laboratory reference range

Date of first enrolment
01/08/2006

Date of final enrolment
01/08/2007

Locations

Countries of recruitment
Netherlands

Study participating centre
Academic Medical Center (AMC)
Amsterdam
Netherlands
1100 DD

Sponsor information

Organisation
Academic Medical Center (AMC) (The Netherlands)

ROR
https://ror.org/03t4gr691

Funder(s)

Funder type
Industry

Funder Name
Merck Sharp and Dohme BV (MSD)

Funder Name
Schering-Plough

Alternative Name(s)

Funding Body Type
Private sector organisation

Funding Body Subtype
For-profit companies (industry)

Location

United States of America

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration