

# Efficacy and safety of ezetimibe in young children with familial hypercholesterolemia

<b>Submission date</b> 07/06/2006	<b>Recruitment status</b> No longer recruiting	<input checked="" type="checkbox"/> Prospectively registered
		<input type="checkbox"/> Protocol
<b>Registration date</b> 07/06/2006	<b>Overall study status</b> Completed	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
<b>Last Edited</b> 07/06/2006	<b>Condition category</b> Nutritional, Metabolic, Endocrine	<input type="checkbox"/> Individual participant data
		<input type="checkbox"/> Record updated in last year

**Plain English summary of protocol**  
Not provided at time of registration

## Contact information

**Type(s)**  
Scientific

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## Additional identifiers

**EudraCT/CTIS number**

**IRAS number**

**ClinicalTrials.gov number**

**Secondary identifying numbers**  
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

### Secondary study design

Randomised controlled trial

### Study setting(s)

Not specified

### Study type(s)

Treatment

## Participant information sheet

### 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 measure**

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

**Secondary outcome measures**

Secondary endpoint will be the effect of 10 mg ezetimibe on inflammatory markers and plant sterols in plasma.

**Overall study start date**

01/08/2006

**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

**Age group**

Child

**Lower age limit**

8 Years

**Upper age limit**

14 Years

**Sex**

Both

**Target number of participants**

70

**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)

**Sponsor details**

P.O. Box 22660

Amsterdam

Netherlands

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**Sponsor type**

University/education

**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

## Publication and dissemination plan

Not provided at time of registration

## Intention to publish date

## Individual participant data (IPD) sharing plan

## IPD sharing plan summary

Not provided at time of registration