An observational study to evaluate the long-term effects of evinacumab treatment in patients with homozygous familial hypercholesterolemia (HoFH)

First published: 20/06/2022

Last updated: 15/10/2024

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/50238

EU PAS number

EUPAS47088

Study ID

50238

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Brazil
- [ ] Canada
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Japan
- [ ] Mexico
- [ ] Netherlands
- [ ] Portugal
- [ ] Spain

Study description

This study is a 5-year observational retrospective cohort study using data collected by the existing European Atherosclerosis Society (EAS) Familial Hypercholesterolemia Studies Collaboration (FHSC) Global Familial Hypercholesterolemia (FH) Registry.

Study status

Ongoing

Research institutions and networks

Institutions

Ultragenyx Germany

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Institution

Contact details

Study institution contact

Study Director Ultragenyx

Study contact

Clinicaltrialtransparency@ultragenyx.com

Primary lead investigator

Study Director Ultragenyx

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/09/2022

Actual: 17/07/2023

Study start date

Planned: 01/07/2023

Actual: 01/07/2023

Data analysis start date

Planned: 01/01/2025

Date of interim report, if expected

Planned: 01/06/2025

Date of final study report
Planned: 30/06/2029

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ultragenyx

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 2 (specific obligation of marketing authorisation)

Regulatory procedure number

EMEA/H/C/005449/0005

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Effectiveness study (incl. comparative)

Main study objective:

To evaluate the safety, disease progression, and pregnancy outcomes in participants with HoFH who are treated with evinacumab.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Registry study of HoFH patients treated with evinacumab in real world setting

Study drug and medical condition

Study drug International non-proprietary name (INN) or common name

EVINACUMAB

Anatomical Therapeutic Chemical (ATC) code

(C10AX17) evinacumab

evinacumab

Medical condition to be studied

Type IIa hyperlipidaemia

Population studied

Age groups

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Pregnant women

Estimated number of subjects

50

Study design details

Outcomes

The study will evaluate long-term safety outcomes, the frequency and outcomes of pregnancy in female participants, changes in the atherosclerosis process over time and frequency of cardiovascular imaging in participants with

HoFH and treated with evinacumab.

Data analysis plan

Descriptive analysis of safety data with annual interim analysis and final report at 5 years.

Data management

Data sources

Data sources (types)

Disease registry

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No