Exploration of Biomarkers in Familial Hypercholesterolemia (FH)

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Primary objective is to explore a panel of biomarkers to support the scientific evaluation of BRN-002 as a potential therapeutic agent in the treatment of FH.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Metabolic and nutritional disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON51891

Source
ToetsingOnline

Brief title
FH-001

Condition

- Metabolic and nutritional disorders congenital

Synonym
hereditary high cholesterol

Research involving
Human

Sponsors and support

Primary sponsor: Beren Therapeutics P.B.C.
Source(s) of monetary or material Support: opdrachtgever

Intervention

Keyword: Biomarkers, Familial Hypercholesterolemia (FH)

Outcome measures

Primary outcome

N/A

Secondary outcome

N/A

Study description

Background summary

Familial Hypercholesterolemia (FH) is a common life-threatening genetic condition that results in high circulating low-density lipoprotein (LDL) containing cholesterol. As a result, FH is associated with early morbidity and mortality, primarily due to rapid and progressive atherosclerosis. A number of gene mutations have been associated with FH. Patients can range from single gene mutation heterozygotes, to multiple gene mutation compound heterozygotes, through to gene mutation homozygotes. The severity of presentation and disease follows this progress of gene mutations from heterozygotes through compound heterozygotes to homozygotes.

The gene mutations most commonly reported are reduction/loss of function in the LDL receptor (LDLR), reduction/loss of function of Apolipoprotein B (APOB), and gain of function in proprotein convertase subtilisin/kexin type 9 (PCSK9). However, a significant number of patients with clinical features consistent with FH have no mutations in these genes.

There are a number of criteria used to diagnose FH depending on region and preferences of the clinician. Finding of a defined mutation(s) is considered definitive. The primary clinical feature used is the fasting LDL-Cholesterol (LDL-C) and total cholesterol levels. Tendon xanthomas are associated with severe FH. Additional factors include patient history of early onset atherosclerosis and a family history of cardiovascular disease.

Study objective

Primary objective is to explore a panel of biomarkers to support the scientific evaluation of BRN-002 as a potential therapeutic agent in the treatment of FH.

Study design

There will be max. two blood collections (55 ml, approximately 11 teaspoons) for subjects. Blood samples will be used for long-term storage and future analyses and will be stored for 10 years.

The study duration will include study sections as follows:
- Screening Visit
- Day 1 Visit (Screening & Baseline Visit may also be performed in one day)

Intervention

One blood sample will be collected on Day 1 (55 ml).

Study burden and risks

Two blood samples (55 ml) and associated risks

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age

Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)

Inclusion criteria

1. Patients clinically diagnosed using regional diagnostic criteria (e.g., Simon Broome, Dutch Lipid Network, MEDPED, etc.) and/or genetically confirmed diagnosis of Familial Hypercholesterolemia (FH)
2. >=12 years of age
3. Triglycerides <= 300 mg/dL
4. Average Fasting LDL-C levels:
o >=190mg/dL within last 30 days for untreated adults
o >=150mg/dL within last 30 days for untreated children
OR
o >=160mg/dL within last 30 days for stably treated patients (adults & children)

Exclusion criteria

1. Conditions or circumstances that, in the opinion of the Investigator, could interfere with or confound study results, or preclude informed consent.
2. Liver transplant recipients.
3. Patients who have undergone partial ileal bypass surgery.
4. Patients with evidence in medical history of chronic HBV infection, HCV infection, or HIV infection
5.Patients who are currently participating or have participated in a clinical trial in which investigational intervention was administered within 12 weeks or 5 half-lives, whichever is longer, of Screening in this clinical study.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Basic science

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	23-12-2021
Enrollment:	25
Type:	Actual

Ethics review

Approved WMO Date:	08-12-2021
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	30-05-2022
Application type:	Amendment
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
CCMO	NL78683.018.21