A Long-term Follow-up Study to Evaluate the Safety and Efficacy of RGX-501

Published: 25-06-2020
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The primary study objective is long-term safety of RGX-501 and secondarily evaluation of the long-term effects of RGX-501 on LDL-C and other lipid parameters.

Ethical review	Approved WMO
Status	Completed
Health condition type	Lipid metabolism disorders
Study type	Observational invasive

Summary

ID

NL-OMON49424

Source
ToetsingOnline

Brief title
Safety and Efficacy of RGX-501

Condition

- Lipid metabolism disorders

Synonym
familial hypercholesterolaemia

Research involving
Human

Sponsors and support

Primary sponsor: REGENXBIO Inc
Source(s) of monetary or material Support: REGENXBIO Inc

Intervention

Keyword: Efficacy, HoFH, RGX-501, Safety

Outcome measures

Primary outcome

The primary objective is to evaluate the long-term safety of RGX-501.

Secondary outcome

Secondary objectives are

* To evaluate the long-term effect of RGX-501 on LDL-C and other lipid

parameters,

and

* To evaluate the long-term impact of RGX-501 on the use of other lipid-lowering

therapies, including apheresis.

Study description

Background summary

RGX-501 is an investigational gene therapy in patients with HoFH. RGX-501 is a liver-directed AAV vector serotype 8 (AAV8) based gene therapy that contains genes that allow expression of functional LDLR in transfected cells. It is anticipated that, following gene therapy administration, hepatocytes will be able to express functional, transgenic LDLRs that can bind to and remove LDL-C from circulation.

Study objective

The primary study objective is long-term safety of RGX-501 and secondarily evaluation of the long-term effects of RGX-501 on LDL-C and other lipid parameters.

Study design

This is a prospective, observational study to evaluate the long-term safety and efficacy after a single administration of RGX-501.

Study burden and risks

The possible risks with joining this study include the risk and discomfort from blood draws. The risks of drawing blood include temporary discomfort from the needle in your arm, fainting, bruising, clotting, and swelling at the needle site and, in rare instances, infection.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

To be eligible to participate in this study, a participant must have previously received RGX-501 in a
separate parent trial, and the participant or participant*s legal guardian(s) is/(are) willing and able to
provide written, signed informed consent after the nature of the study has been explained, prior to any

research-related procedures.

Exclusion criteria

see inclusion criteria

Study design

Design

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

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	27-11-2020
Enrollment:	2
Type:	Actual

Ethics review

Approved WMO Date:	25-06-2020
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	12-08-2020
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)

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	EUCTR2019-004496-39-NL
ClinicalTrials.gov	NCT04080050
CCMO	NL73955.000.20