16 June 2021

Sponsor:

REGENXBIO Inc.
9600 Blackwell Road, Suite 210
Rockville, MD 20850
United States

Protocol no.: FHGT002

Protocol Title: AAV8-mediated Low-Density Lipoprotein Receptor (LDLR) Gene Replacement in Subjects with Homozygous Familial Hypercholesterolemia (HoFH)

EudraCT no.: 2016-001446-25

The FHGT002 trial entitled "AAV8-mediated Low-Density Lipoprotein Receptor (LDLR) Gene Replacement in Subjects with Homozygous Familial Hypercholesterolemia (HoFH)" was prematurely ended.

This was a 104-week, Phase 1/2a, multicenter, open-label, single arm, dose escalation study of AAV8.TBG.hLDLR in adults with a clinical presentation consistent with homozygous familial hypercholesterolemia (HoFH) with mutations in both low-density lipoprotein receptor (LDLR) alleles. Safety was the primary focus, with a secondary focus on clinical response to AAV8.TBG.hLDLR. Approximately 12 subjects were planned to be enrolled into 1 of 3 possible dose Cohorts. Although safety was acceptable to proceed to Cohort 3 dosing, REGENXBIO Inc. decided to terminate the study prior to subject enrollment for business reasons; therefore, only 9 subjects were enrolled in the study. Following REGENXBIO Inc. decision, all active subjects were transitioned to a long-term follow up study and are continuing to be followed.

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