Study to Evaluate Safety, Tolerability and Efficacy of Inclisiran in Children With Homozygous Familial Hypercholesterolemia

Last Update: Apr 11, 2025
Two Part (Double-blind Inclisiran Versus Placebo [Year 1] Followed by Open-label Inclisiran [Year 2]) Randomized Multicenter Study to Evaluate Safety, Tolerability, and Efficacy of Inclisiran in Children (2 to Less Than 12 Years) With Homozygous Familial Hypercholesterolemia and Elevated LDL-cholesterol
ClinicalTrials.gov Identifier:
NCT06597006
Novartis Reference Number:CKJX839C12304
See if you Pre-qualify
All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

This is a pivotal phase III study designed to evaluate safety, tolerability, and efficacy of inclisiran in children (aged 2 to \<12 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDLC). This is a two-part (1 year double-blind inclisiran versus placebo / 1 year open-label inclisiran) multicenter study designed to evaluate safety, tolerability, and efficacy of inclisiran in children (aged 2 to \<12 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C) on stable standard of care background lipid-lowering therapy.

Condition
Familial Hypercholesterolemia - Homozygous
Phase
Phase3
Overall Status
Recruiting
Number of Participants
9
Start Date
Feb 28, 2025
Completion Date
Apr 15, 2029
Gender
All
Age(s)
2 Years - 11 Years (Child)

Interventions

Drug

Inclisiran

Inclisiran (inclisiran sodium 300 mg subcutaneous (s.c.) for participants with body weight ≥23 kg, inclisiran sodium 180 mg s.c. for participants with body weight \
Drug

Placebo

Sterile normal saline (0.9% sodium chloride in water for subcutaneous injection)

Eligibility Criteria

Inclusion Criteria:

* Male or female participants, 2 to \<12 years of age at screening
* HoFH diagnosed by genetic confirmation

- Note: Participants with known null (negative) mutations in both LDLR alleles are not eligible (see also exclusion criteria)
* Fasting LDL-C \>130 mg/dL (3.4 mmol/L) at screening
* On an optimal dose of statin (investigator's discretion), unless statin intolerant, with or without other lipid-lowering therapy (e.g. ezetimibe)
* Participants on lipid-lowering therapies (such as e.g. statins, ezetimibe) must be on a stable dose for ≥30 days before screening with no planned medication or dose changes during study participation
* Participants on a documented regimen of LDL-apheresis for ≥ 3 months before screening will be allowed to continue the apheresis during the study, if needed. The apheresis schedule/settings/duration must be stable prior to screening, are not allowed to change during the double-blind period of the trial and must permit that an apheresis coincides with each study visit.

Exclusion Criteria:

* Documented evidence of a null (negative) mutation in both LDLR alleles
* Previous treatment (within 90 days of screening) with monoclonal antibodies directed towards PCSK9
* History of poor response to therapy with any monoclonal antibody directed towards PCSK9 (e.g. \<15% reduction in LDL-C)
* Treatment with mipomersen or lomitapide (within 5 months of screening)
* Secondary hypercholesterolemia, e.g. hypothyroidism or nephrotic syndrome
* Heterozygous familial hypercholesterolemia (HeFH)
* Body weight (at the screening and/or randomization (Day 1) visit) \<16 kg for participants 6 to \<12 years (at screening) or \<11 kg for participants 2 to \<6 years (at screening)
* Active liver disease defined as any known current infectious, neoplastic, or metabolic pathology of the liver or unexplained alanine aminotransferase (ALT), aspartate aminotransferase (AST) elevation \>3x ULN, or total bilirubin elevation \>2x ULN (except patients with Gilbert's syndrome)
* Pregnant or nursing females
* Recent and/or planned use of other investigational medicinal products or devices

China

Novartis Investigative Site

Recruiting

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Source URL: *https://prod1.novartis.com/clinicaltrials/study/nct06597006*

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1. https://clinicaltrials.gov/ct2/show/NCT06597006
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