Study to Evaluate Efficacy and Safety of Inclisiran in Children With Heterozygous Familial Hypercholesterolemia

Status: RECRUITING

Eligibility Criteria

Age: 6 years to 11 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Male or female participants, 6 to \<12 years of age at screening * HeFH diagnosed either by genetic testing or on phenotypic criteria * Fasting LDL-C \>130 mg/dL (3.4 mmol/L) at screening * For participants 8 to \<12 years, on an optimal dose of statin (investigator's discretion) unless statin intolerant, with or without other lipid-lowering therapy (e.g. ezetimibe). For participants \<8 years, the use of background lipid-lowering treatment is based on investigator's discretion. * Participants on lipid-lowering therapies (such as statin and/or e.g. ezetimibe) must be on a stable dose for ≥30 days before screening with no planned medication or dose changes during study participation.

Exclusion Criteria:

* Previous treatment (within 90 days of screening) with monoclonal antibodies directed towards PCSK9 * Secondary hypercholesterolemia, e.g. hypothyroidism or nephrotic syndrome * Homozygous familial hypercholesterolemia (HoFH) * Body weight \<16 kg at the screening and/or randomization (Day 1) visit * 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

Conditions & Interventions

Interventions:
DRUG: Inclisiran, DRUG: Placebo
Conditions:
Familial Hypercholesterolemia - Heterozygous
Keywords:
Heterozygous familial hypercholesterolemia (HeFH),, LDL-cholesterol (LDL-C),, Children,, pediatric,, small interfering ribonucleic acid (siRNA),, inclisiran,, Familial Hypercholesterolemia,, Heterozygous FH,, Hypercholesterolemia,, Lipoprotein(a),, Hyperlipidemia,, Dyslipidemia,, Heart Failure,, Cardiovascular Diseases,, Cholesterol,, Aortic Stenosis

More Information

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