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

Last Update: May 02, 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 (6 to Less Than 12 Years) With Heterozygous Familial Hypercholesterolemia and Elevated LDL- Cholesterol
ClinicalTrials.gov Identifier:
NCT06597019
Novartis Reference Number:CKJX839C12303
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 6 to \<12 years) with heterozygous familial hypercholesterolemia (HeFH) 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 6 to \<12 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C) on stable standard of care background lipid-lowering therapy.

Condition
Familial Hypercholesterolemia - Heterozygous
Phase
Phase3
Overall Status
Recruiting
Number of Participants
51
Start Date
Dec 09, 2024
Completion Date
Apr 15, 2029
Gender
All
Age(s)
6 Years - 11 Years (Child)

Interventions

Drug

Inclisiran

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

Argentina

Novartis Investigative Site

Recruiting

Caba,Buenos Aires,C1181ach,Argentina

Austria

Novartis Investigative Site

Recruiting

Salzburg,5020,Austria

Novartis Investigative Site

Recruiting

Wien,1090,Austria

China

Novartis Investigative Site

Recruiting

Beijing,100029,China

Czech Republic

Novartis Investigative Site

Recruiting

Praha 5,150 06,Czech Republic

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Recruiting

Praha,12808,Czech Republic

France

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Recruiting

Marseille,13885,France

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Recruiting

Paris,75571,France

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Recruiting

Nantes Cedex 1,44093,France

Germany

Novartis Investigative Site

Recruiting

Freiburg,79106,Germany

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Recruiting

Hannover,30173,Germany

Greece

Novartis Investigative Site

Recruiting

Ioannina,GR,455 00,Greece

Hungary

Novartis Investigative Site

Recruiting

Budapest,1026,Hungary

Israel

Novartis Investigative Site

Recruiting

Jerusalem,9112001,Israel

Novartis Investigative Site

Recruiting

Ramat Gan,52621,Israel

Italy

Novartis Investigative Site

Recruiting

Milano,MI,20162,Italy

Novartis Investigative Site

Recruiting

Verona,VR,3712,Italy

Malaysia

Novartis Investigative Site

Recruiting

Kuala Lumpur,Wilayah Persekutuan,50586,Malaysia

Poland

Novartis Investigative Site

Recruiting

Gdansk,80 952,Poland

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Recruiting

Lodz,93-338,Poland

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Recruiting

Bialystok,15-274,Poland

Portugal

Novartis Investigative Site

Recruiting

Lisboa,1649 035,Portugal

Spain

Novartis Investigative Site

Recruiting

Barcelona,08041,Spain

Novartis Investigative Site

Recruiting

Cadiz,Andalucia,11009,Spain

Novartis Investigative Site

Recruiting

Elche,Alicante,03203,Spain

Novartis Investigative Site

Recruiting

Malaga,Andalucia,29010,Spain

Novartis Investigative Site

Recruiting

Sevilla,Andalucia,41013,Spain

Novartis Investigative Site

Recruiting

Pamplona,Navarra,31008,Spain

Taiwan

Novartis Investigative Site

Recruiting

Taipei,11217,Taiwan

Turkey

Novartis Investigative Site

Recruiting

Ankara,06500,Turkey

United States

UC San Francisco Medical Center

Recruiting

San Francisco,California,94143,United States

Laura Dapkus Humphries

Phone: +1 415 476 8338
Email: laura.dapkus@ucsf.edu

Martin Thelin

Excel Medical Clinical Trials LLC

Recruiting

Boca Raton,Florida,33434,United States

Hilariann Tribble

Phone: +1 561 756 8206
Email: htribble@flourishresearch.com

Rasha Youssef

Worldwide Contacts

If the location of your choosing does not feature any contact detail, please reach out using the information below.

Novartis Pharmaceuticals

Phone: +41613241111
Email:

Novartis Pharmaceuticals

Phone: 1-888-669-6682
Email: novartis.email@novartis.com

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7. tel:+41613241111
8. mailto:
9. tel:1-888-669-6682
10. mailto:novartis.email@novartis.com