Long-term Safety and Tolerability of Inclisiran in Participants With HeFH or HoFH Who Have Completed the Adolescent ORION-16 or ORION-13 Studies

22/11/2024 05:07:44

Main Information

Primary registry identifying number
LBCTR2023045322

Protocol number
CKJX839C12001B

MOH registration number

Study registered at the country of origin
Yes

Study registered at the country of origin: Specify

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency

Primary sponsor
Novartis Pharma AG

Primary sponsor: Country of origin
Novartis Pharma AG

Date of registration in primary registry
18/07/2024

Date of registration in national regulatory agency

Public title
Long-term Safety and Tolerability of Inclisiran in Participants With HeFH or HoFH Who Have Completed the Adolescent ORION-16 or ORION-13 Studies

Acronym

Scientific title
An Open-label, Single Arm, Multicenter Extension Study to Evaluate Long-term Safety and Tolerability of Inclisiran in Participants With Heterozygous or Homozygous Familial Hypercholesterolemia Who Have Completed the Adolescent ORION-16 or ORION-13 Studies (VICTORION-PEDS-OLE)

Acronym

Brief summary of the study: English
The purpose of this open-label, single arm, multicenter extension study is to evaluate the long-term safety and tolerability of inclisiran in participants with HeFH or HoFH who have completed the ORION -16 or ORION-13 studies

Brief summary of the study: Arabic
دراسة تمديد مفتوحة التسمية ومتعددة المراكز من مجموعة واحدة لتقييم سلامة إنكليسيران وقدرة تحمّله على المدى الطويل لدى مشاركين مصابين بفرط كوليسترول الدم العائلي متغاير الزيجوت أو متماثل الزيجوت أنجزوا دراسة أوريون-16 (ORION-16) أو دراسة أوريون-13(ORION-13) للمراهقين (VICTORION-PEDS-OLE)

Health conditions/problem studied: Specify
Heterozygous or Homozygous Familial Hypercholesterolemia

Interventions: Specify
Drug: Inclisiran
Inclisiran sodium 300mg (equivalent to 284mg inclisiran*) in 1.5mL solution administered subcutaneously in pre-filled syringe
Other Name: KJX839

Key inclusion and exclusion criteria: Inclusion criteria

Key inclusion:

1- Male and female participants with a diagnosis of HeFH or HoFH who completed the ORION-16 or ORION-13 studies
2- Per investigator's clinical judgment, participant derived benefit from treatment with inclisiran in the ORION-16 or ORION-13 studies

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

12

Key inclusion and exclusion criteria: Age maximum

17

Key inclusion and exclusion criteria: Exclusion criteria

Key exclusion:

1- Participants who in the feeder inclisiran ORION-16 and ORION-13 studies either screen failed or permanently discontinued from the treatment/study for any reason or had serious safety or tolerability issues related to inclisiran treatment
2- Any uncontrolled or serious disease, or any medical, physical, or surgical condition, that may either interfere with participation in the clinical study or interpretation of clinical study results, and/or put the participant at significant risk

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Therapy

Trial scope: Specify scope

N/A

Study design: Allocation

Single Arm Study

Study design: Masking

Open (masking not used)

Study design: Control

N/A

Study phase

3

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Single

Study design: Specify assignment

N/A

IMP has market authorization

Yes, Worldwide

IMP has market authorization: Specify

European Union, United Arab Emirates, Great Britain

Name of IMP

Inclisiran

Year of authorization

Month of authorization

Type of IMP

Others

Pharmaceutical class

Cholesterol-lowering small interfering ribonucleic acid (siRNA) that inhibits the production of proprotein convertase subtilisin/kexin type 9 (PCSK9)

Therapeutic indication

Heterozygous or Homozygous Familial Hypercholesterolemia

Therapeutic benefit

Treatment

Study model
N/A

Study model: Explain model
N/A

Study model: Specify model
N/A

Time perspective
N/A

Time perspective: Explain time perspective
N/A

Time perspective: Specify perspective
N/A

Target follow-up duration

Target follow-up duration: Unit

Number of groups/cohorts

Biospecimen retention
Samples without DNA

Biospecimen description
Blood samples collected will be analyzed at Medpace Laboratories, central Lab

Target sample size
4

Actual enrollment target size

Date of first enrollment: Type
Anticipated

Date of first enrollment: Date
30/08/2023

Date of study closure: Type
Anticipated

Date of study closure: Date
30/08/2026

Recruitment status
Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan
Yes

IPD sharing statement description
Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations.

This trial data availability is according to the criteria and process described on www.clinicalstudydatarequest.com

Additional data URL

https://clinicaltrials.gov/ct2/show/record/NCT05682378

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
clinical trials.gov	NCT05682378

Sources of Monetary or Material Support

Name
Novartis Pharma AG

Secondary Sponsors

Name
NA

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Selim Jambart	Beirut	Lebanon	009613406001	sjambart@dm.net.lb	Hotel Dieu De France
Scientific	Hind Khairallah	Sin El Fil	Lebanon	009611512002 Ext. 271 E	hind.khairallah@fattal.com.lb	khalil Fattal et Fils s.a.l
Scientific	Hala Tfayli	Beirut	Lebanon	0096171729759	ht31@aub.edu.lb	American University of Beirut Medical Center, Hamra, Lebanon

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu De France	Selim Jambart	Endocrinology	Approved
American University of Beirut Medical Center	Hala Tfayli	Endocrinology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	30/03/2023	Sami Richa	cue@usj.edu.lb	009611421229
American University of Beirut Medical Center	06/10/2023	Rami Mahfouz	rm11@aub.edu.lb	009611350000 ext 5445

Countries of Recruitment

Name
Lebanon
Brazil
Canada
France
Germany
Greece
Hungary
Italy
Netherlands
Norway
Poland
Russian Federation
Slovenia
Spain
Switzerland
United States of America

Health Conditions or Problems Studied

Condition	Code	Keyword
Heterozygous or homozygous familial hypercholesterolemia	Hyperlipidaemia, unspecified (E78.5)	Heterozygous or homozygous familial hypercholesterolemia

Interventions

Intervention	Description	Keyword
Consenting, IMP administration, Laboratory testing, Imaging	Consenting, IMP administration, Laboratory testing, Imaging	Consenting, IMP administration, Laboratory testing, Imaging

Primary Outcomes

Name	Time Points	Measure
Number of participants with treatment-emergent adverse events (TEAEs) and serious adverse events (TESAEs)	Time Frame: From Day 1 in the study up to the end of study visit; up to 1080 days	Safety and tolerability: TEAEs, TESAEs (incidence, severity, relationship to study drug and discontinuation due to TEAEs)

Key Secondary Outcomes

Name	Time Points	Measure
Percentage and absolute change in LDL-C from baseline in the feeder study to end of study	Time Frame: Baseline (of feeder study) and Day 1080	Evaluate the long-term effect of inclisiran (from baseline of feeder study to end of study) in lowering LDL-C

Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files