



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

13/08/2025 00:27:47

## 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

21/08/2023

### 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 تحمله على المدى الطويل لدى مشاركين - أو دراسة أوربيون (ORION-16) مصابين بفرط كوليسترول الدم العائلي متغاير الزيجوت أو متماثل الزيجوت أنجزوا دراسة أوربيون-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: Specify model**

N/A

**Time perspective**

N/A

**Time perspective: Specify perspective**

N/A

**Target follow-up duration**

**Number of groups/cohorts**

**Biospecimen retention**

Samples without DNA

**Target sample size**

4

**Date of first enrollment: Type**

Anticipated

**Date of study closure: Type**

Anticipated

**Recruitment status**

Pending

**Date of completion**

**IPD sharing statement plan**

Yes

**Additional data URL**

**Study model: Explain model**

N/A

**Time perspective: Explain time perspective**

N/A

**Target follow-up duration: Unit**

**Biospecimen description**

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

**Actual enrollment target size**

**Date of first enrollment: Date**

30/08/2023

**Date of study closure: Date**

30/08/2026

**Recruitment status: Specify**

**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](http://www.clinicalstudydatarequest.com)



<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

## 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



## 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	00961421229

## 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