



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

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## 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**

21/11/2023

**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: 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](http://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 |

## 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**