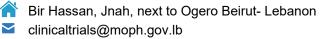
REPUBLIC OF LEBANON Lebanon Clinical Trials Registry

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

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lain Information	
Primary registry identifying number	Protocol number
LBCTR2021034776	CKJX839C12301
MOH registration number	
Study registered at the country of origin	Study registered at the country of origin: Specify
Yes	
Type of registration	Type of registration: Justify
Prospective	N/A
Date of registration in national regulatory agency	
Primary sponsor	Primary sponsor: Country of origin
Novartis Pharma Services inc.	Novartis Pharmaceuticals
Date of registration in primary registry	Date of registration in national regulatory agency
23/05/2021	
Public title	Acronym
Study to Evaluate Efficacy and Safety of Inclisiran in Adolescents With Heterozygous Familial Hypercholesterolemia	
Scientific title	Acronym
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 Adolescents (12 to Less Than 18 Years) With Heterozygous Familial Hypercholesterolemia and Elevated LDL-cholesterol (ORION-16)	
Brief summary of the study: English	
This is a pivotal phase III study designed to evaluate safety, tolerability, and efficacy of inclisiran in adolescents with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C).	
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 adolescents with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C) on stable standard of care background lipid-lowering therapy. The primary objective is to demonstrate superiority of inclisiran compared to placebo in reducing LDL-C (percent change) at Day 330.	
Brief summary of the study: Arabic	
ة متعددة المراكز ، عشوانيَّة التوزيع من قسمين (إنكليسيران مزدوج التعمية مقابل الدواء الوهمي [السنة مائلي18 إلى أقل من 12]) لتقييم سلامة إنكليسيران وقدرة تحمّله وفعاليَّته لدى المراهقين (من 2السنة ] (ORION-16) )16متغاير الزيجوت وبارتفاع الكوليسترول الضار (أوريون-	
Health conditions/problem studied: Specify	



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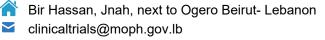
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Heterozygous Familial Hypercholesterolemia		
Interventions: Specify		
Drug: Inclisiran Drug: Placebo		
Key inclusion and exclusion criteria: Inclusion criteria		
Heterozygous Familial Hypercholesterolemia (HeFH) diagnosed either by ger Fasting LDL-C >130 mg/dL (3.4 mmol/L) at screening Fasting triglycerides <400 mg/dL (4.5 mmol/L) at screening On maximally tolerated dose of statin (investigator's discretion) with or without screening Estimated glomerular filtration rate (eGFR) >30 mL/min/1.73 m2 at screening		
Key inclusion and exclusion criteria: Gender	Key inclusion and exclusion cri	teria: Specify gender
Both		
Key inclusion and exclusion criteria: Age minimum	Key inclusion and exclusion cri	teria: Age maximum
12	17	
Key inclusion and exclusion criteria: Exclusion criteria		
Homozygous familial hypercholesterolemia (HoFH) Active liver disease Secondary hypercholesterolemia, e.g. hypothyroidism or nephrotic syndrome Major adverse cardiovascular events within 3 months prior to randomization Previous treatment with monoclonal antibodies directed towards PCSK9 (with Recent and/or planned use of other investigational medicinal products or dev Other protocol-defined inclusion/exclusion criteria may apply	nin 90 days of screening)	
Type of study		
Interventional		
Type of intervention	Type of intervention: Specify ty	ре
Pharmaceutical	N/A	
Trial scope	Trial scope: Specify scope	
Therapy	N/A	
Study design: Allocation	Study design: Masking	
Randomized controlled trial	Blinded (masking used)	
Study design: Control	Study phase	
Placebo	3	
Study design: Purpose	Study design: Specify purpose	
Treatment	N/A	
Study design: Assignment	Study design: Specify assignme	ent
Parallel	N/A	
IMP has market authorization	IMP has market authorization: S	Specify
Yes, Worldwide	European Union, United Arab Emi	irates, Great Britain
Name of IMP	Year of authorization	Month of authorization
inclisiran		
Type of IMP		
Others		
Pharmaceutical class		



cholesterol-lowering small interfering ribonucleic acid (siRNA) that inhibits the production of proprotein

cholesterol-lowering small interfering ribonucleic acid (siRNA) that inhibits the production of proprotein convertase subtilisin/kexin type 9				
Therapeutic indication				
heterozygous familial hypercholesterolemia (HeFH) and elevated low densi (LDL-C)	ty lipoprotein cholesterol			
Therapeutic benefit				
to demonstrate superiority of inclisiran compared to placebo in reducing LD Day 330.	L-C (percent change) at			
Study model	Study model: Explain model			
N/A	N/A			
Study model: Specify model				
N/A				
Time perspective	Time perspective: Explain time perspective			
N/A	N/A			
Time perspective: Specify perspective				
N/A				
Target follow-up duration	Target follow-up duration: Unit			
Number of groups/cohorts				
Number of groups/conorts				
Biospecimen retention	Biospecimen description			
Samples with DNA**	Blood samples collected will be analyzed at Medpace Laboratories, central lab			
Target sample size	Actual enrollment target size			
4				
Date of first enrollment: Type	Date of first enrollment: Date			
Anticipated	30/04/2021			
Date of study closure: Type	Date of study closure: Date			
Anticipated	16/08/2023			
Recruitment status	Recruitment status: Specify			
Pending				
Date of completion				
29/07/2021				
IPD sharing statement plan	IPD sharing statement description			
Yes				





# Lebanon Clinical Trials Registry

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/NCT04652726?cond=heterozygous+familial+hypercholesterolemia&draw=2&rank=2

Admin comments

Trial status

Approved

Secondary Identifying Numbers		
Full name of issuing authority	Secondary identifying number	
NCT04652726	Clinical trials.gov	

Sources of Monetary or Material Support		
Name		
Novartis Pharma Services inc.		

Secondary Sponsors	
Name	
NA	

Contact for Public/Scientific Queries						
Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Selim Jambart	Beirut	Lebanon	961 3 406 001	sjambart@dm.ne t.lb	Hotel Dieu De France
Scientific	Hind Khairallah	Sinelfil	Lebanon	01512002# 271	Hind.khairallah@ fattal.com.lb	Khalil Fattal et Fils s.a.l.





Centers/Hospitals Involved in the Study				
Center/Hospital nameName of principles investigatorPrinciples investigator specialityEthical 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	21/12/2020	Sami Richa	cue@usj.edu.lb	961421229

Countries of Recruitment
Name
Lebanon
Australia
Germany
Hungary
Norway
Spain
United States of America

Health Conditions or Problems Studied			
Condition Code Keyword			
heterozygous familial hypercholesterolemia	Hyperlipidaemia, unspecified (E78.5)	heterozygous familial hypercholesterolemia	

Interventions				
Intervention	Description	Keyword		
Informed Consent/assent form discussion; Inclusion/exclusion assessment; physical examination; neurological examination; blood and urine samples collection; IMP dispensation	Informed Consent/assent form discussion; Inclusion/exclusion assessment; physical examination; neurological examination; blood and urine samples collection; IMP dispensation	ICF, IMP , Lab tests, physical exams		



# Primary OutcomesNameTime PointsMeasurePercentage (%) change in low-density lipoprotein cholesterol<br/>(LDL-C)baseline to Day 330baseline to Day 330Demonstrate superiority of inclisiran compared to placebo in<br/>reducing LDL-C [percent change]Day 330 (Year 1)Day 330 (Year 1)

### **Key Secondary Outcomes**

They become any outcomes		
Name	Time Points	Measure
Time-adjusted % change in LDL-C from baseline	Baseline, after Day 90 up to Day 330	Baseline, after Day 90 up to Day 330
Absolute change in LDL-C from baseline to Day 330	Baseline and Day 330	Baseline and Day 330
% change in apolipoprotein B (Apo B), lipoprotein (a) [Lp(a)], non-high density lipoprotein cholesterol (non-HDL-C), and total cholesterol from baseline to Day 330	Baseline and Day 330	Baseline and Day 330
% change and absolute change in LDL-C from baseline up to Day 720	Baseline, up to Day 720	Baseline, up to Day 720
% change and absolute change in other lipoproteins and lipid parameters	Baseline, up to Day 720	Baseline, up to Day 720
% change and absolute change in proprotein convertase subtilisin/kexin type 9 (PCSK9)	Baseline, up to Day 720	Baseline, up to Day 720



# Trial Results Summary results Study results globally Date of posting of results summaries 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