Helios-B : A Study to Evaluate Vutrisiran in Patients with Transthyretin Amyloidosis with Cardiomyopathy

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Main Information

Primary registry identifying number
LBCTR2020104517

Protocol number
ALN-TTRSC02-003

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
29/05/2020

Primary sponsor
Alnylam Pharmaceuticals, Inc.

Primary sponsor: Country of origin
USA

Date of registration in primary registry
23/05/2022

Date of registration in national regulatory agency
29/05/2020

Public title
Helios-B : A Study to Evaluate Vutrisiran in Patients with Transthyretin Amyloidosis with Cardiomyopathy

Acronym
HELIOS-B

Scientific title
Helios-B: A Phase 3, Randomized, Doubleblind, Placebo-controlled, Multicenter Study to Evaluate the Efficacy and Safety of Vutrisiran in Patients with Transthyretin Amyloidosis with Cardiomyopathy (ATTR Amyloidosis with Cardiomyopathy)

Acronym
HELIOS-B

Brief summary of the study: English
This study will evaluate the efficacy and safety of vutrisiran 25 mg administered subcutaneously (SC) once every 3 months (q3M) compared to placebo in patients with ATTR amyloidosis with cardiomyopathy.

Brief summary of the study: Arabic
اشهر مقارنة مع الدواء الوهمي في3 مغم يعطى في شكل حقنة تحت الجلد مرة كل 25ستقوم هذه الدراسة بتقييم فعالية وسلامة فوتريزيران المرضى الذين يعانون من الداء النشواني مع اعتلال عضلة القلب.

Health conditions/problem studied: Specify
Transthyretin Amyloidosis (ATTR) With Cardiomyopathy

Interventions: Specify
Arm:
Experimental: Vutrisiran 25 mg
Participants will receive vutrisiran 25 mg administered subcutaneously (SC) once every 3 months (q3M) during the double-blind period.
Assigned Intervention:
Drug: Vutrisiran
Vutrisiran 25 mg will be administered by SC injection q3M.
Other Name: ALN-TTRSC02

Placebo Comparator: Placebo
Participants will receive placebo during the double-blind period.
Assigned Intervention:
Drug: Sterile Normal Saline (0.9% NaCl)
Sterile normal saline (0.9% NaCl) will be administered by SC injection q3M.

Key inclusion and exclusion criteria: Inclusion criteria

Inclusion criteria:
1. Has a documented diagnosis of transthyretin (ATTR) amyloidosis with cardiomyopathy, classified as either hereditary ATTR (hATTR) Amyloidosis with cardiomyopathy or wild-type ATTR (wtATTR) amyloidosis with cardiomyopathy meeting pre-specified diagnostic criteria.
2. Has medical history of heart failure (HF) with at least 1 prior hospitalization for HF OR clinical evidence of HF.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

18

Key inclusion and exclusion criteria: Age maximum

85

Key inclusion and exclusion criteria: Exclusion criteria

Exclusion criteria:
1. Has known primary amyloidosis or leptomeningeal amyloidosis
2. Has New York Heart Association (NYHA) Class IV heart failure
3. Has NYHA Class III heart failure AND is at high risk based on pre-specified criteria
4. Has a polyneuropathy disability (PND) Score IIIa, IIIb, or IV at the Screening visit
5. Has estimated glomerular filtration rate (eGFR) <30 mL/min/1.73 m^2
6. Has received prior TTR-lowering treatment
7. Has other non-TTR cardiomyopathy, hypertensive cardiomyopathy, cardiomyopathy due to valvular heart disease, or cardiomyopathy due to ischemic heart disease

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

Randomized controlled trial

Study design: Masking

Blinded (masking used)

Study design: Control

Placebo

Study phase

3

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Parallel

Study design: Specify assignment

N/A

IMP has market authorization

No

IMP has market authorization: Specify

Name of IMP

Vutrisiran (ALN-TTRSC02)

Year of authorization

Month of authorization

Type of IMP

Gene therapy

Pharmaceutical class

Synthetic RNA interference (RNAi) therapeutic molecule

Therapeutic indication

Transthyretin Amyloidosis (ATTR) With Cardiomyopathy

Therapeutic benefit

Vutrisiran utilizes RNAi to prevent the synthesis of both wt and mutant TTR in the liver, the primary source of circulating TTR.
TTR reduction with vutrisiran will beneficially impact disease progression in patients with ATTR amyloidosis with cardiomyopathy.

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 with DNA**

Biospecimen description

Biological specimens will be collected, may include DNA, RNA, or biochemical metabolite assessments as they relate to disease progression, efficacy or safety.
The biospecimen repository will also include residual material from routine samples (safety laboratory samples, PK samples, etc.) that are obtained during the study.

Target sample size

600

Actual enrollment target size

Date of first enrollment: Type

Actual

Date of first enrollment: Date

28/05/2021

Date of study closure: Type

Actual

Date of study closure: Date

22/09/2021

Recruitment status

Complete

Recruitment status: Specify

Date of completion

31/07/2021

IPD sharing statement plan

No

IPD sharing statement description

Not Applicable

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Food and Drug Administration	NCT04153149
Eudract Number	2019-003153-28

Sources of Monetary or Material Support

Name
Alnylam Pharmaceuticals, Inc

Secondary Sponsors

Name
NA

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Aziz El Zoghbi	MCT-CRO	Lebanon	+961 71 008 269	zog_az@mctcro.com	Director of Country Oversight and Management Africa, Levant and GCC
Scientific	Alnylam Clinical Trial Information Line Central Contact Person	Not applicable	United States of America	1-877-ALNYLAM	clinicaltrials@alnylam.com	Alnylam Pharmaceuticals, Inc
Scientific	Alnylam Clinical Trial Information Line Central Contact Backup	Not applicable	United States of America	1-877-256-9526	Not Applicable	Alnylam Pharmaceuticals, Inc
Scientific	Jean El Cheikh	American University of Beirut Medical Center	Lebanon	+961 71 407 447	je46@aub.edu.lb	Principal Investigator

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
American University of Beirut Medical Center	Jean El Cheikh	Hematology/Oncology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
American University of Beirut Medical Center	12/10/2020	Jean El Cheikh	je46@aub.edu.lb	+96171407447

Countries of Recruitment

Name
Lebanon
France
United States of America
Argentina
Australia
Austria
Belgium
Brazil

Canada
Colombia
Bulgaria
Denmark
Germany
Ireland
Italy
Czech Republic
Mexico
Norway
Japan
Peru
Republic of Moldova
Poland
Netherlands
Portugal
Spain
Sweden
United Kingdom
Malaysia
Taiwan
Romania
Croatia
Estonia
Finland
Costa Rica
Greece

Hungary
Latvia
Lithuania
Saudi Arabia
Slovakia
Slovenia
Jordan

Health Conditions or Problems Studied

Condition	Code	Keyword
Transthyretin Amyloidosis with Cardiomyopathy	Heart failure (I50)	Cardiomyopathy

Interventions

Intervention	Description	Keyword
Experimental: Vutrisiran 25 mg	Participants will receive vutrisiran 25 mg administered subcutaneously (SC) once every 3 months (q3M) during the double-blind period	Vutrisiran, SC, q3M
Placebo Comparator: Placebo	Participants will receive placebo during the double-blind period.	Placebo

Primary Outcomes

Name	Time Points	Measure
Composite Endpoint of All-Cause Mortality and Recurrent Cardiovascular (CV) Events (CV Hospitalizations and Urgent Heart Failure [HF] Visits)	[Time Frame: 30-36 months]	All-cause mortality and recurrent CV events (CV hospitalizations and urgent HF visits) will be compared between treatment groups using an Andersen-Gill model.

Key Secondary Outcomes

Name	Time Points	Measure
Change from Baseline in 6-Minute Walk Test (6-MWT) at Month 30	[Time Frame: Baseline, Month 30]	Change from baseline in 6-minute walk test (6-MWT)
Change from Baseline in the Kansas City Cardiomyopathy Questionnaire Overall Summary (KCCQ-OS) at Month 30	[Time Frame: Baseline, Month 30]	Change from baseline in the Kansas City Cardiomyopathy Questionnaire Overall Summary (KCCQ-OS)
Change from Baseline in Mean Left Ventricular (LV) Wall Thickness by Echocardiographic Assessment at month 30	[Time Frame: Baseline, Month 30]	Change from Baseline in Mean Left Ventricular (LV) Wall Thickness by Echocardiographic Assessment
Change from Baseline in Global Longitudinal Strain by Echocardiographic Assessment at month 30	[Time Frame: Baseline, Month 30]	Change from Baseline in Global Longitudinal Strain by Echocardiographic Assessment
Composite Endpoint of All-Cause Mortality and Recurrent Allcause Hospitalizations and Urgent HF Visits	[Time Frame: 30-36 months]	Composite Endpoint of All-Cause Mortality and Recurrent All-cause Hospitalizations and Urgent HF Visits using an Andersen-Gill model.
All-cause Mortality	[Time Frame: 30-36 months]	All-cause mortality
Rate of Recurrent CV Events (CV Hospitalizations and Urgent HF Visits)	[Time Frame: 30-36 months]	Recurrent CV hospitalizations
Change from Baseline in N-terminal prohormone B-type Natriuretic Peptide (NTproBNP) at month 30	[Time Frame: Baseline, Month 30]	Change from Baseline in N-terminal prohormone Btype Natriuretic Peptide (NTproBNP)

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