A Phase 2 Open-Label Study to Evaluate the Safety and Efficacy of DCR-PHXC in Patients With Primary Hyperoxaluria Type 1 or 2 and Severe Renal Impairment, With or Without Dialysis

22/11/2024 04:03:26

Main Information

Primary registry identifying number
LBCTR2022125202

Protocol number
DCR-PHXC-204

MOH registration number
NCT04580420

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
21/01/2021

Primary sponsor
Dicerna Pharmaceuticals

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
29/02/2024

Date of registration in national regulatory agency
21/01/2021

Public title
A Phase 2 Open-Label Study to Evaluate the Safety and Efficacy of DCR-PHXC in Patients With Primary Hyperoxaluria Type 1 or 2 and Severe Renal Impairment, With or Without Dialysis

Acronym

Scientific title
A Phase 2 Open-Label Study to Evaluate the Safety and Efficacy of DCR-PHXC in Patients With Primary Hyperoxaluria Type 1 or 2 and Severe Renal Impairment, With or Without Dialysis

Acronym
PHYOX7

Brief summary of the study: English
This is a repeat dose, uncontrolled, open-label, Phase 2 study of DCR-PHXC in patients
with primary hyperoxaluria type 1 (PH1) or type 2 (PH2) and severe renal impairment,
with or without dialysis.

Brief summary of the study: Arabic
هذه جرعة متكررة ، غير خاضعة للرقابة ، مفتوحة التسمية ، دراسة المرحلة 2 لـ DCR-PHXC في المرضى
مع فرط أوكسالات البول الأولي من النوع 1 (PH1) أو النوع 2 (PH2) ، والضعف الكلوي الشديد
مع أو بدون غسيل الكلى.

Health conditions/problem studied: Specify
Primary Hyperoxaluria Type 1 (PH1)
Primary Hyperoxaluria Type 2 (PH2)
Kidney Diseases
Urologic Diseases
Genetic Disease

Interventions: Specify

Drug: DCR-PHXC
Multiple fixed doses of DCR-PHXC by subcutaneous (SC) injection.
Other Name: Nedosiran

Key inclusion and exclusion criteria: Inclusion criteria

Key inclusion criteria include:
•Genetically confirmed PH1 or PH2
•Estimated glomerular filtration rate (eGFR) < 30 mL/min normalized to 1.73 m2 body surface area (BSA)
•Mean of 2 Pox values > 20 µmol/L during Screening
•For participants receiving hemodialysis or peritoneal dialysis, total duration of hemodialysis or peritoneal dialysis must be less than or equal to 18 months and stable dialysis regimen for at least 2 weeks prior to Screening
•Willing to adhere to a low oxalate diet and avoid high doses of vitamin C

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

0

Key inclusion and exclusion criteria: Age maximum

99

Key inclusion and exclusion criteria: Exclusion criteria

Key exclusion criteria include:
•Hepatic transplantation, prior to or planned in the 6 months from Day 1. Renal transplantation planned in the 6 months from Day 1. Prior renal transplantation is allowed.
•Documented evidence of clinical manifestations of systemic oxalosis

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

2

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Single

Study design: Specify assignment

N/A

IMP has market authorization

No

IMP has market authorization: Specify

Name of IMP

Nedosiran

Year of authorization

Month of authorization

Type of IMP

Others

Pharmaceutical class

A synthetic double-stranded (hybridized duplex) ribonucleic acid (RNA) oligonucleotide conjugated to N-acetyl-D-galactosamine (GalNAc) amino-sugar residues.

Therapeutic indication

Primary Hyperoxaluria.

Therapeutic benefit

At present, no therapies are approved by regulatory authorities for the treatment of patients with PH. DCR-PHXC treatment has the potential benefit to reduce or eliminate the excess oxalate production in the liver and thus avoid the need for a combined liver and kidney transplantation in patients not already on renal replacement therapy.

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

None retained

Biospecimen description

blood and urine samples.

Target sample size

30

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

30/03/2023

Date of study closure: Type

Anticipated

Date of study closure: Date

30/11/2026

Recruitment status

Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan

No

IPD sharing statement description

Participants will be assigned a unique identifier by the Sponsor. Any participant records or datasets that are transferred to the Sponsor will contain the identifier only; participant names or any information which would make the participant identifiable will not be transferred.

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
US NCT Number	NCT04580420

Sources of Monetary or Material Support

Name
Dicerna Pharmaceuticals, Inc. 75 Hayden Ave. Lexington, MA 02421 US (617) 621-8097

Secondary Sponsors

Name
N/A

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Chadi Safa	ebanon. Baabda. Chiah. Ain el remeneh	Lebanon	009617125 1819	csafa@ctifact.com	CTI
Scientific	Chebl Mourani	Alfred Naccache Blvd, External Viewing Tower, Floor 4, Room 9403	Lebanon	03 290090	cheblmourani@gmail.com	HDF

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu De France Hospital	Dr Chebl Mourani	Pediatric Nephrologist	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	15/11/2022	Nancy El Alam	nancy.alam@usj.edu.lb	+961 1 421 000

Countries of Recruitment

Name
United States of America
Germany
France
Spain
Italy
Lebanon
United Kingdom
United Arab Emirates
Romania

Health Conditions or Problems Studied

Condition	Code	Keyword
Primary Hyperoxaluria	2-Propanol (T51.2)	N/A

Interventions

Intervention	Description	Keyword
Nedosiran	IMP	N/A

Primary Outcomes

No Outcomes

Key Secondary Outcomes

No Outcomes

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