Long Term Extension Study in Patients With Primary Hyperoxaluria

21/11/2024 17:12:20

Main Information

Primary registry identifying number
LBCTR2020124677

Protocol number
DCR-PHXC-301

MOH registration number
NCT04042402

Study registered at the country of origin
No

Study registered at the country of origin: Specify
Study registered in clinicaltrials.gov

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency
02/08/2019

Primary sponsor
Dicerna Pharmaceuticals, Inc.

Primary sponsor: Country of origin
USA

Date of registration in primary registry
20/02/2021

Date of registration in national regulatory agency
02/08/2019

Public title
Long Term Extension Study in Patients With Primary Hyperoxaluria

Acronym

Scientific title
An Open-Label Roll-Over Study to Evaluate the Long-Term Safety and Efficacy of DCR-PHXC Solution for Injection (Subcutaneous Use) in Patients With Primary Hyperoxaluria

Acronym
“PHYOX3”

Brief summary of the study: English
The proposed study is designed to provide patients previously enrolled in Phase 1 and 2 studies of DCR-PHXC long-term access to DCR-PHXC, and to evaluate the long-term safety and efficacy of DCR-PHXC in patients with PH.

Brief summary of the study: Arabic
تم تصميم الدراسة المقترحة لتزويد المرضى المسجلين سابقًا في دراسات المرحلتين الأولى والثانية من DCR-PHXC بالوصول طويل الأمد إلى DCR-PHXC ، ولتقييم سلامة وفعالية DCR-PHXC على المدى الطويل في مرضى PH.

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:

•Participant successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC.

OR Participant is the sibling of a participant who successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC. Siblings must be younger than 18 years of age and must have genetically confirmed PH.

•For participants rolling over from a multidose study of DCR-PHXC, enrollment should occur within a window of 25 to 60 days from the last dose of study intervention. Estimated GFR at screening ≥ 30 mL/min normalized to 1.73 m2 body surface area (BSA), calculated using Chronic Kidney Disease Epidemiology Collaboration (CKD EPI) formula in participants aged ≥ 18 years (Levey & Stevens, 2010), or the formula by Schwartz in participants aged 6 to 16 years (Schwartz et al., 2009; National Kidney Foundation, 2002). In Japan, the formula by Uemura et al. will be used for participants aged 6 to 17 years (Uemura et al., 2014).

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

6

Key inclusion and exclusion criteria: Age maximum

99

Key inclusion and exclusion criteria: Exclusion criteria

Key exclusion criteria include:
• Renal or hepatic transplantation; prior or planned within the study period
• Current dialysis
• Documented evidence of clinical manifestations of systemic oxalosis (including pre-existing retinal, heart, or skin calcifications, or history of severe bone pain, pathological fractures, or bone deformations).

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Other

Trial scope: Specify scope

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

No

IMP has market authorization: Specify

Name of IMP

DCR PHXC

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

50

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

01/02/2021

Date of study closure: Type

Anticipated

Date of study closure: Date

30/12/2023

Recruitment status

Other

Recruitment status: Specify

Enrolling by Invitation

Date of completion

30/12/2023

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	NCT04042402

Sources of Monetary or Material Support

Name
Dicerna pharmaceuticals inc. 75 Hayden Avenue Suite 400 Lexington, MA 02421, USA

Secondary Sponsors

Name
NA

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Chadi Safa	lebanon. Baabda. Chiah. Ain el remeneh	Lebanon	009617125 1819	Chadi.safa@clinart.net	Clinart
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
1.Hotel Dieu de France	Chebl Mourani	Pediatric Nephrologist	Approved
2.Saint George University Hospital	Pauline Abu jaoude	Nephrologist	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	30/03/2020	Nancy Choukair Alam	nancy.alam@usj.edu.lb	: +961 1 421 000
Saint George Hospital University Medical Center	09/07/2020	Sandra Berberi	smberbari@stgeorgehospital.org	+961 1 44 16 30

Countries of Recruitment

Name
France
Netherlands
Germany
United Kingdom
United States of America
Lebanon
Spain
Italy
Australia
Canada
Japan

Health Conditions or Problems Studied

Condition	Code	Keyword
Primary Hyperoxaluria	2-Propanol (T51.2)	Kidney Diseases

Interventions

Intervention	Description	Keyword
DCR-PHXC	Multiple fixed doses of DCR-PHXC by subcutaneous (SC) injection	Nedosiran

Primary Outcomes

Name	Time Points	Measure
To evaluate the effect of DCR PHXC on estimated glomerular filtration rate	Annual change from baseline	estimated glomerular filtration rate

Key Secondary Outcomes

Name	Time Points	Measure
The incidence and severity of treatment-emergent adverse events (TEAE) and SAEs associated with abnormal 12 lead electrocardiogram (ECG) readings	TEAEs and SAEs are evaluated monthly for 3 years	Electrocardiogram (ECG)

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