A Phase 2 Open-Label Multicenter Study to Evaluate the Safety, Pharmacokinetics, and Efficacy of Nedosiran in Pediatric Patients from Birth to 5 Years of Age with Primary Hyperoxaluria and Relatively Intact Renal Function

09/04/2025 15:24:57

Main Information

Primary registry identifying number
LBCTR2021104866

Protocol number
DCR-PHXC-203

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
04/11/2019

Primary sponsor
Dicerna Pharmaceuticals

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
27/01/2022

Date of registration in national regulatory agency
04/11/2019

Public title
A Phase 2 Open-Label Multicenter Study to Evaluate the Safety, Pharmacokinetics, and Efficacy of Nedosiran in Pediatric Patients from Birth to 5 Years of Age with Primary Hyperoxaluria and Relatively Intact Renal Function

Acronym

Scientific title
A Phase 2 Open-Label Multicenter Study to Evaluate the Safety, Pharmacokinetics, and Efficacy of Nedosiran in Pediatric Patients from Birth to 5 Years of Age with Primary Hyperoxaluria and Relatively Intact Renal Function

Acronym

Brief summary of the study: English

This is a Phase 2, multi-dose (3.5 mg/kg), open-label, single-arm, uncontrolled, multicenter study of nedosiran in pediatric participants (birth to 5 years of age) with genetically confirmed PH type 1 (PH1), type 2 (PH2), or type 3 (PH3) with relatively intact renal function based upon eGFR and serum creatinine.
Participants will receive monthly SC doses (3.5 mg/kg) of nedosiran over 6 months.
The primary objective of this study is to characterize the safety of nedosiran in pediatric participants (birth to 5 years of age) with PH1, PH2, and PH3. The secondary objective of this study is to characterize the efficacy of nedosiran in pediatric participants (birth to 5 years of age) with PH1, PH2, and PH3. The efficacy of nedosiran in lowering Uox will be assessed via monthly spot urine samples.
Participants completing this study may be eligible for long-term treatment with nedosiran in Study DCR-PHXC-301.

Brief summary of the study: Arabic

هذه مرحلة 2) ، جرعات متعددة عن3.5 مجم / كجم) ، علامة مفتوحة ، ذراع واحدة ، غير خاضعة للرقابة ، دراسة متعددة المراكز
2النوع ، (PH1) 1مؤكد وراثيًا من النوع PH سنوات) مع نوع5في المشاركين في طب الأطفال (من الولادة حتى سن nedosiran
(PH2) ، 3أو النوع (PH3) مع وظيفة كلوية سليمة نسبيًا على أساس معدل الترشيح الكبيبي (eGFR) وكرياتينين المصل.
أشهر6على مدى nedosiran مجم / كجم) من3.5(SC سيحصل المشاركون على جرعات شهرية من .
PH1 و سنوات من العمر) مع5عند الأطفال المشاركين (من الولادة إلى nedosiran الهدف الأساسي من هذه الدراسة هو توصيف سلامة
PH2 و PH3. سنوات من العمر) مع5في الأطفال المشاركين (من الولادة إلى nedosiran الهدف الثانوي لهذه الدراسة هو توصيف فعالية
PH1 و PH2 و PH3. من خلال عينات البول الموضعية الشهرية Uox في خفض nedosiran سيتم تقييم فعالية.
قد يكون المشاركون الذين أكملوا هذه الدراسة مؤهلين للعلاج طويل الأمد باستخدام nedosiran في دراسة DCR-PHXC-301.

Health conditions/problem studied: Specify

Primary Hyperoxaluria Type 1, 2 & 3.

Interventions: Specify

monthly SC doses (3.5 mg/kg) of nedosiran over 6 months.

Key inclusion and exclusion criteria: Inclusion criteria

Key inclusion criteria include
• Estimated glomerular filtration rate (eGFR) at Screening ≥ 30 mL/min normalized to 1.73 m2 body surface area (BSA).
• Average spot Uox-to-creatinine ratio at Screening above 2 times the 95th percentile for age based on Matos et al, 1999:
o > 0.44 mol/mol in participants < 6 months
o > 0.34 mol/mol in participants from 6 months to <12 months
o > 0.26 mol/mol in participants 12 months to < 2 years
o > 0.20 mol/mol in participants from 2 to < 3 years and
o > 0.16 mol/mol in participants from 3 to 5 years

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

5

Key inclusion and exclusion criteria: Exclusion criteria

Key exclusion criteria include
• Renal or hepatic transplantation (prior or planned within the study period)
• Plasma oxalate (Pox) > 30 µmol/L at Screening
• Documented evidence of clinical manifestations of severe systemic oxalosis (including preexisting 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

Safety

Trial scope: Specify scope

N/A

Study design: Allocation

Single Arm Study

Study design: Masking

Open (masking not used)

Study design: Control

Dose comparison

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

IMP has market authorization: Specify

No

Name of IMP

Nedosiran

Year of authorization

Month of authorization

Type of IMP

Others

Pharmaceutical class

DCR-PHXC consists of the drug substance (DCR-L1360) in WFI) DCR-L1360 is a synthetic double-stranded (hybridized duplex) RNA oligonucleotide conjugated to GalNAc aminosugar residues. After SC administration, the GalNAc sugars conjugated to the RNA oligonucleotide bind to asialoglycoprotein receptors (ASGR) to deliver DCR-L1360 to hepatocytes.

Therapeutic indication

DCR-PHXC (Nedosiran sodium) reduces the level of mRNA encoding the dominant form of the LDH enzyme, specifically, the LDHA isoenzyme. Lactate dehydrogenase catalyzes the cytosolic conversion of glyoxylate to oxalate in the liver and this biochemical reaction is believed to be critical for oxalate generation for all 3 genetic forms of PH.

Therapeutic benefit

DCR-PHXC (Nedosiran sodium) reduces the level of mRNA encoding the dominant form of the LDH enzyme, specifically, the LDHA isoenzyme. Lactate dehydrogenase catalyzes the cytosolic conversion of glyoxylate to oxalate in the liver and this biochemical reaction is believed to be critical for oxalate generation for all 3 genetic forms of PH.

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

blood samples, urine samples and buccal cells.

Target sample size

5

Actual enrollment target size

5

Date of first enrollment: Type

Date of first enrollment: Date

Anticipated

06/12/2021

Date of study closure: Type

Anticipated

Date of study closure: Date

02/01/2023

Recruitment status

Pending

Recruitment status: Specify

Date of completion

09/12/2022

IPD sharing statement plan

No

IPD sharing statement description

N/A

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Dicerna Pharmaceuticals	N/A

Sources of Monetary or Material Support

Name
Dicerna Pharmaceuticals

Secondary Sponsors

Name
Premier Research

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Sarah Kharsa	Beirut	Lebanon	+96181209199	sarah.kharsa@clinart.net	Clinart MEA
Scientific	Nancy Choucair	Beirut	Lebanon	+9611421000	nancy.alam@usj.edu.lb	Hotel Dieu De France Ethics Committee

Centers/Hospitals Involved in the Study

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

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	07/07/2021	Nancy Choucair	nancy.alam@usj.edu.lb	01-421000

Countries of Recruitment

Name
Lebanon
United States of America
United Kingdom
France
Poland
Turkey

Health Conditions or Problems Studied

Condition	Code	Keyword
Primary Hyperoxaluria	Nephrotic syndrome, other (N04.8)	hyperoxaluria

Interventions

Intervention	Description	Keyword
Nedosiran	DCR-PHXC 170 mg/mL Solution for Injection	Nedosiran

Primary Outcomes

Name	Time Points	Measure
To characterize the safety of nedosiran in neonates, infants, and young children with PH and relatively intact renal function based upon eGFR and serum creatinine	6 months	Change from Baseline in 12-lead ECG, physical examination findings, vital sign assessments, and clinical laboratory tests

Key Secondary Outcomes

Name	Time Points	Measure
To assess the efficacy of nedosiran in neonates, infants, and young children with PH and relatively intact renal function based upon eGFR and serum creatinine	6 months	Percent and absolute change from Baseline to Month 6 in spot urinary oxalate-to-creatinine ratio

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