A Natural History Study of Patients with Genetically Confirmed Primary Hyperoxaluria Type 3 with a History of Stone Events

11/09/2025 04:57:38

Main Information

Primary registry identifying number
LBCTR2021054742

Protocol number
DCR-PHXC-502

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

Primary sponsor
Dicerna Pharmaceuticals, Inc.

Primary sponsor: Country of origin
United States

Date of registration in primary registry
09/12/2022

Date of registration in national regulatory agency

Public title
A Natural History Study of Patients with Genetically Confirmed Primary Hyperoxaluria Type 3 with a History of Stone Events

Acronym

Scientific title
A Natural History Study of Patients with Genetically Confirmed Primary Hyperoxaluria Type 3 with a History of Stone Events

Acronym

Brief summary of the study: English
This is a natural history study of adults, adolescents, and children (≥ 2 years of age) with
genetically confirmed primary hyperoxaluria type 3 (PH3) who have a history of stone
events during the last 3 years and/or the presence of pre-existing stones detected by renal
ultrasound at Screening.

Brief summary of the study: Arabic
مع تاريخ حالات الحصوات3دراسة التاريخ الطبيعي للمرضى المصابين بفرط أوكسالات البول الأولي المؤكد وراثيًا من النوع

Health conditions/problem studied: Specify
Primary Hyperoxaluria type 3

Interventions: Specify
this is a non-interventional study

Key inclusion and exclusion criteria: Inclusion criteria
Key inclusion criteria include
• Genetically confirmed PH3
• History of stone events (defined as presence of calcifications in the urinary tract and/or kidney, their relative location, and the number and size of stones) during

the last 3 years and/or presence of pre-existing stones detected by renal ultrasound at Screening
• Uox > 0.7 mmol/24 hours normalized to 1.73 m2 BSA
• eGFR at Screening ≥ 30 mL/min
• Able to accommodate 24-hour urine collection

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

2

Key inclusion and exclusion criteria: Age maximum

85

Key inclusion and exclusion criteria: Exclusion criteria

Exclusion Criteria
Participants are excluded from the study if any of the following criteria apply:
Medical Conditions
1. Prior hepatic transplantation; or planned transplantation within the study period
2. Currently receiving dialysis or anticipating requirement for dialysis during the study period

Type of study

Observational

Type of intervention

N/A

Type of intervention: Specify type

N/A

Trial scope

N/A

Trial scope: Specify scope

N/A

Study design: Allocation

N/A

Study design: Masking

N/A

Study design: Control

N/A

Study phase

N/A

Study design: Purpose

N/A

Study design: Specify purpose

N/A

Study design: Assignment

N/A

Study design: Specify assignment

N/A

IMP has market authorization

IMP has market authorization: Specify

Name of IMP

Year of authorization

Month of authorization

Type of IMP

Pharmaceutical class

Therapeutic indication

Therapeutic benefit

Study model

Other

Study model: Explain model

Natural History of Patients with PH3 and a History of Stone Events

Study model: Specify model

natural history study

Time perspective

Retrospective

Time perspective: Explain time perspective

This is a non-interventional study
that will last up to 2 years.

Time perspective: Specify perspective

N/A

Target follow-up duration

1

Target follow-up duration: Unit

year

Number of groups/cohorts

0

Biospecimen retention

Samples with DNA**

Biospecimen description

blood and urine samples

Target sample size

480

Actual enrollment target size

1

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

15/06/2021

Date of study closure: Type

Anticipated

Date of study closure: Date

01/01/2024

Recruitment status

Pending

Recruitment status: Specify

Date of completion

15/06/2023

IPD sharing statement plan

No

IPD sharing statement description

N/A, this is an observational trial.

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Hotel Dieu De France Ethics Committee	N/A

Sources of Monetary or Material Support

Name
Premier Research CRO

Secondary Sponsors

Name
Dicerna Pharmaceuticals INC.

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Sarah Kharsa	Beirut	Lebanon	+81-209199	sarah.kharsa@clinart.net	Clinart MEA
Scientific	Chadi Safa	Beirut	Lebanon	+9613210603	chadi.safa@clinart.net	Clinart MEA

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu De France University Hospital	Dr. Chebl Mourani	Pediatrics	Approved
Saint George University Hospital Medical Center	Dr. Pauline Abou Jaoude	Pediatrics	Pending

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	02/02/2021	Nancy Alam	nancy.alam@usj.edu.lb	+961 1 421 000 ext 2335

Countries of Recruitment

Name
Lebanon
United States of America
Canada
United Kingdom
France
Germany
Poland

Health Conditions or Problems Studied

Condition	Code	Keyword
Primary Hyperoxaluria	Nephrotic syndrome (N04)	N/A

Interventions

No Interventions

Primary Outcomes

Name	Time Points	Measure
The objective of this study is to collect data on stone formation and the degree of nephrocalcinosis in patients (≥ 2 years of age) with genetically confirmed PH3 and relatively intact renal function and to explore the potential relationship between Uox and new stone formation.	4	stone formation

Key Secondary Outcomes

Name	Time Points	Measure
new stone formation and degree of nephrocalcinosis	N/A	N/A

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