



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

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## 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 ( $\geq$  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 الحصوات 3 دراسة 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: Specify model

natural history study

## Time perspective

Retrospective

## Time perspective: Specify perspective

N/A

## Target follow-up duration

1

## Number of groups/cohorts

0

## Biospecimen retention

Samples with DNA\*\*

## Study model: Explain model

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

## Time perspective: Explain time perspective

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

## Target follow-up duration: Unit

year

## 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 ( $\geq 2$ 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