PHYOS: An observational study of patients with Primary Hyperoxaluria type1

Published: 15-12-2015
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Primary Objective• To collect data regarding changes in oxalate, glycolate, and other metabolites over time in subjects with primary hyperoxaluria type 1 (PH1) who may be considered for clinical trials of DCR PH1Secondary Objectives• To collect data...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Other condition
Study type	Observational invasive

Summary

ID

NL-OMON42592

Source
ToetsingOnline

Brief title
DCR-PH1-501

Condition

- Other condition

Synonym
PH1

Health condition

autosomal recessive disorders

Research involving
Human

Sponsors and support

Primary sponsor: Dicerna Pharmaceuticals, Inc
Source(s) of monetary or material Support: Dicerna Pharmaceutics

Intervention

Keyword: Observational, Primary Hyperoxaluria type 1

Outcome measures

Primary outcome

N.A.

Secondary outcome

N.A.

Study description

Background summary

PHYOS is an observational study to collect data regarding changes in oxalate, glycolate, and other biochemical parameters in patients with PH1 who may be considered for upcoming clinical trials of DCR-PH1, a novel experimental agent being developed for the treatment of PH1.

Study objective

Primary Objective
• To collect data regarding changes in oxalate, glycolate, and other metabolites over time in subjects with primary hyperoxaluria type 1 (PH1) who may be considered for clinical trials of DCR PH1

Secondary Objectives
• To collect data regarding clinical manifestations associated with PH1
• To collect data regarding fluid intake using a diary for self-reporting by subjects
• To collect data regarding quality of life (QOL) in patients with PH1

Study design

This is an observational study: prospective data collection will continue up to

6 months per subject, during which subjects will be seen in clinic at Screening, Day 1, Month 4 and Month 6 (± 15 days).

Study burden and risks

No investigational drugs will be administered.

Collection of blood samples may cause mild pain, redness, bruising and or irritation at the injection site.

Contacts

Public
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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- Diagnosis of PH1, confirmed by genotyping for mutations in the AGXT gene.
- Urine oxalate excretion >=0.7 mmol per 1.73 m2 body surface area (BSA) in 24 hours.
- Estimated glomerular filtration rate (eGFR) >=40 mL/min per 1.73 m2 BSA.

Exclusion criteria

- Prior renal and/or hepatic transplantation, or patients undergoing dialysis.
- Pregnancy or lactation at the time of screening or enrollment.
- Any significant illness, organ system dysfunction, or other condition that, in the opinion of the Investigator, would interfere with the subject*s ability to comply with the protocol requirements, including the ability to attend all visits and undergo all assessments.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Basic science

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	14-01-2016
Enrollment:	7
Type:	Actual

Ethics review

Approved WMO

Date:	15-12-2015
Application type:	First submission
Review commission:	METC Amsterdam UMC

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL54393.018.15