DCR-PHXC Phase 1 Clinical Trial Overview

Dicerna Pharmaceuticals initiated the Phase 1 PHYOX clinical trial of DCR-PHXC in Q4 2017, when the first normal healthy volunteer was dosed. The Company dosed the first patient in Q2 2018. Dicerna is studying DCR-PHXC as a potential treatment option for patients with *all* forms of primary hyperoxaluria (PH).

Single ascending-dose study divided into two groups

COMPLETE

Group A
Normal Healthy Volunteers

Placebo-controlled
Single-blind
Single-center

Eligibility Criteria
Includes but is not limited to:

- Male or female participants between 18 and 55 years of age
- Body mass index (BMI) 19.0 to 32 kg/m^2
- Non-smokers

IN PROGRESS

Group B
Patients with either PH1 or PH2

Open-label
Multi-center

Eligibility Criteria
Includes but is not limited to:

- Male or female, 6 years of age or older
- Minimum body weight of 25 kg
- Diagnosed with PH1 or PH2

41 → Total number of participants in the study
6 to 55 years old → Age of subjects

Primary Endpoint	• Evaluate the safety and tolerability of single doses of DCR-PHXC in both groups
Secondary Endpoints	• Evaluate the pharmacodynamic (PD) effects of single doses of DCR-PHXC on biochemical markers including, changes in urine oxalate concentrations • Characterize the pharmacokinetics (PK) of single doses of DCR-PHXC in normal healthy volunteers and patients with PH

For more information on the trial, including additional inclusion or exclusion criteria, please view the Frequently Asked Questions page of **PHYOXTrials.com** or visit **clinicaltrials.gov** and enter the identifier number NCT03392896.

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