Dicerna	Objective	Inclusion Criteria	Exclusion Criteria	Time Points	Sites	For more Info.
PHYOX 1 (Completed)	**Study of DCR-PHXC-101 in Normal Healthy Volunteers (HV) and Patients with Primary Hyperoxaluria**	**Group A (HVs)** Willing and able to provide informed consent and comply with study requirements. or female subjects between 18 5 years of age, inclusive. Subject must have a body mass index (BMI) 19.0 to 32 kg/m2, inclusive. Non-smokers, at least 1-month tobacco free, and willing to remain tobacco free through end of study (EOS). Women of childbearing potential must have a negative pregnancy test, cannot be breastfeeding, and must be willing to use contraception. **Group B (PH1 and PH2 patients)** Willing and able to provide informed consent and comply with study requirements. Male or female, at least 6 years of age. Minimum body weight of 25 kg. Genetic confirmation of PH1 and PH2 disease. Meet the 24 hour urine oxalate excretion requirements. Estimated glomerular filtration rate (eGFR) ≥30 mL/min/1.73 m2. If taking Vitamin B6 (pyridoxine), must have been on stable regimen for at least 4 weeks.	**Group A (HVs)** Presence of any medical condition, including but not limited to: Severe intercurrent illness, known causes of active liver disease. Routine or chronic use of more than 3 grams of acetaminophen (Tylenol) daily. History of kidney stones. Use of any investigational agent within 90 days before the first dose of study medication. History of donation of more than 450 mL of blood within 90 days prior to dosing in the clinical research center or planned donation less than 30 days after receiving Investigational Medicinal Product (IMP). Plasma or platelet donation within 7 days of dosing and through EOS. History of reactions to an oligonucleotide-based therapy. Males with female partners who are planning to attempt to become pregnant during this study or within 90 days after last dosing of IMP. Plasma or platelet donation within 7 days of dosing and through EOS. **Group B (PH1 and PH2 patients**) Prior renal and/or hepatic transplantation. Currently receiving dialysis. Participation in any clinical study where they received an investigational agent within 4 months before enrollment. Presence of any medical condition, including but not limited to: Severe	**Time Frame:** **Group A (HVs)** 29 days **Group B (PH 1 and PH2 patients)** 57 days	**France** Bron **Germany** Bonn **Netherlands** Amsterdam **United Kingdom** Birmingham Wales	ClinicalTrials.gov (PHYOX 1) **Email:** medicalinfo@dicerna.com

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			intercurrent illness, known causes of active liver disease. Liver function test (LFT) abnormalities. History of reactions to an oligonucleotide-based therapy.			
PHYOX 2 (Recruiting)	**A Study to Evaluate DCR-PHXC in Children and Adults with PH type 1 and PH type 2**	Capable and willing to provide written informed consent or assent Documented diagnosis of PH1 or PH2, confirmed by genotyping Must meet the 24 hour urine oxalate excretion requirements Less than 20% variation between the two 24-hour urinary creatinine excretion values derived from the two 24-hour urine collections in the screening period Estimated GFR at screening ≥ 30 mL/min normalized to 1.73 m2 BSA	Renal or hepatic transplantation (prior or planned within the study period) Currently on dialysis or anticipated requirement for dialysis during the study period Plasma oxalate >30 µmol/L Documented evidence of clinical manifestations of systemic oxalosis (including pre-existing retinal, heart, or skin calcifications, or history of severe bone pain, pathological fractures, or bone deformations) Use of an RNA interference (RNAi) drug within the last 6 months Participation in any clinical study in which you received an investigational medicinal product (IMP) within 4 months before Screening Liver function test (LFT) abnormalities: Alanine aminotransferase (ALT) and/or aspartate aminotransferase (AST) >1.5 times upper limit of normal (ULN) for age and gender Inability or unwillingness to comply with study procedures	**Time Frame:** 6 months	**France** Bron Paris **Germany** Bonn Heidelberg **Italy** Roma **Netherlands** Amsterdam **United Kingdom** Birmingham London **Israel** Jerusalem **Poland** Biatystok **Romania** Bucharest **Spain** Barcelona Santa Cruz	ClinicalTrials.gov (PHYOX 2) **Email:** medicalinfo@dicerna.com
PHYOX 3 (enrolling by invitation)	**The proposed study is designed to provide patients previously enrolled in Phase 1 and 2 studies of DCR-PHXC long-term access to DCR-PHXC, and to evaluate the long-term safety and**	Participant successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC. OR Participant is the sibling of a participant who successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC. Siblings must be	Renal or hepatic transplantation (prior or planned within the study period) Currently dialysis Documented evidence of clinical	**Time Frame:** 3 years	**France** Bron Paris **Germany** Bonn Heidelberg **Netherlands** Amsterdam	ClinicalTrials.gov (PHYOX 3) **Email:** medicalinfo@dicerna.com

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	efficacy of DCR-PHXC in patients with PH.	younger than 18 years of age and must have genetically confirmed PH. For participants rolling over from a multidose study of DCR-PHXC, enrollment should occur within a window of 25 to 60 days from the last dose of study intervention. Estimated GFR at screening ≥ 30 mL/min normalized to 1.73 m2 body surface area (BSA), calculated using Chronic Kidney Disease Epidemiology Collaboration (CKD EPI) formula in participants aged ≥ 18 years (Levey & Stevens, 2010), or the formula by Schwartz in participants aged 6 to 16 years (Schwartz et al., 2009; National Kidney Foundation, 2002). In Japan, the formula by Uemura et al. will be used for participants aged 6 to 17 years (Uemura et al., 2014).	manifestations of systemic oxalosis		**United Kingdom** Birmingham London	
PHYOX 4 (not yet recruiting)	**The DCR-PHXC-104 study is designed to assess the safety, tolerability, and pharmacological parameters of a single dose of DCR-PHXC in Primary**	Genetically confirmed PH3 24-hour Uox excretion ≥ 0.7 mmol (adjusted per 1.73 m^2 body surface area [BSA] in participants < 18 years of age) on both assessments conducted in the screening period Less than 20% variation between the two 24-hour urinary creatinine excretion values (mmol/kg/24 hours) in the screening period Estimated glomerular filtration rate (eGFR) at screening ≥ 30 mL/min, normalized to 1.73 m^2 BSA History of at least one stone event within the last 12 months.	Documented evidence of clinical manifestations of systemic oxalosis (including pre-existing retinal, heart, or skin calcifications, or history of severe bone pain, pathological fractures, or bone deformations) Plasma oxalate > 30 µmol/L	**Time Frame:** 85 days	**France** Bron Paris **Germany** Bonn Heidelberg **Netherlands** Amsterdam **United Kingdom** London	ClinicalTrials.gov (PHYOX 4) **Email:** medicalinfo@dicerna.com
PHYOX 7 (not yet recruiting)	**The aim of this study is to evaluate DCR-PHXC in participants with PH1 or PH2 and severe renal impairment, with or without dialysis.**	Documented diagnosis of PH1 or PH2, confirmed by genotyping Estimated GFR at Screening <30mL/min normalized to 1.73m^2 BSA Plasma Oxalate >30µmol/L For participants receiving dialysis, total duration must be less than 18 months Male or Female Male participants: A male participant with a female partner of childbearing potential must agree to use contraception during the	Prior hepatic transplantation; or scheduled transplantation within 6 months of Day 1. Prior renal transplantation is allowed. Documented evidence of severe systemic oxalosis, defined as overt signs of bone oxalate deposition in a plain x-ray of the left hand, as evidenced by large diffuse metaphyseal bands Presence of any condition or comorbidities that would interfere with	**Time Frame:** Up to 4 years	TBD	ClinicalTrials.gov (PHYOX 7) **Email:** medicalinfo@dicerna.com

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		treatment period and for at least 12 weeks after the last dose of study intervention and refrain from donating sperm during this period. Female participants: A female participant is eligible to participate if she is not pregnant, not breastfeeding, and at least one of the following conditions applies: Not a woman of childbearing potential (WOCBP). OR A WOCBP who agrees to follow the contraceptive guidance during the treatment period and for at least 12 weeks after the last dose of study intervention. Contraceptive use by men or women should be consistent with local regulations regarding the methods of contraception for those participating in clinical studies. Participant (and/or participant's parent or legal guardian if participant is a minor [defined as patient <18 years of age, or younger than the age of majority according to local regulations]) is capable of giving signed informed consent, which includes compliance with the requirement and restrictions listed in the informed consent form (ICF) and in the protocol.	study compliance or data interpretation or potentially impact patient safety Use of an RNAi drug, other DCR-PHXC, within the last 6 months History of reactions to an oligonucleotide-based therapy Participation in any clinical study in which they received an investigational medicinal product (IMP) other than DCR-PHXC within 4 months before Screening. Liver function test abnormalities: ALT and/or AST >1.5 × ULN for age and gender Positive anti-double-stranded deoxyribonucleic acid (anti-dsDNA) antibody test at Screening Positive urine drug screen (to include at minimum: amphetamines, barbiturates, cocaine, opiates, and benzodiazepines). Urine drug screening is not required for participants ≤ 12 years of age. Exclusion for a positive screen is at the discretion of the Investigator. hypersensitivity to DCR-PHXC or any of its ingredients Inability or unwillingness to comply with the specified study procedures, including the lifestyle considerations			
PHYOX OBX (not yet recruiting)	A natural history trial that will evaluate the association between urinary oxalate levels and stone formation rate. This study will be undertaken for the purposes of providing support for a PH3 indication	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	Prior or planned liver transplant within study period Currently receiving dialysis or anticipating dialysis during study period	**Time Frame:** 2 years		ClinicalTrials.gov (PHYOX OB) **Email:** medicalinfo@dicerna.com

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		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	Unwillingness to comply with study procedures Younger than 2 years old			
The parameters for the following future studies are yet to be determined						
PHYOX 8	Study of DCR-PHXC in children aged 0 to 5 years with PH1 and PH2					**Email:** medicalinfo@dicerna.com
PHYOX 9	An additional study supporting PH3 patients					**Email:** medicalinfo@dicerna.com