



Dicerna's Primary Hyperoxaluria Development Program for 12th International Workshop on Primary Hyperoxaluria

Frequently Asked Questions

Dicerna is providing this information on its drug development efforts as a courtesy to the primary hyperoxaluria community. For more information on the DCR-PHXC program, please visit www.dicerna.com. Please consult your physician regarding any medical decisions and for questions regarding specific clinical trials.

1. Who is Dicerna?

Dicerna Pharmaceuticals, Inc. is a biopharmaceutical company focused on the discovery and development of innovative ribonucleic acid interference (RNAi)-based therapeutics for diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company is using its proprietary GalXC™ RNAi technology platform to build a broad product pipeline in these core therapeutic areas. DCR-PHXC, the lead investigational product candidate in Dicerna's pipeline of therapies targeting rare diseases of the liver, is being developed for the treatment of primary hyperoxaluria (PH). Dicerna is based in Cambridge, Massachusetts.

2. What do the DCR-PHXC data that were presented at the 12th International Workshop on Primary Hyperoxaluria mean for the patient community?

Dicerna presented new preclinical data demonstrating the potential utility of DCR-PHXC, Dicerna's investigational therapy for PH. The research demonstrates how DCR-PHXC inhibits the lactate dehydrogenase A (*LDHA*) gene, which Dicerna has identified as potentially being an optimal therapeutic target in patients with PH.

In contrast, research findings showed that targeting the enzyme glycolate oxidase GO – a common target of investigational therapies for PH, including Dicerna's earlier IV-administered program for PH1 – does not appear to have the potential to treat PH2 or idiopathic PH.

The DCR-PHXC data demonstrate potent, durable and precise knockdown of *LDHA* in animal models of PH1, PH2 and idiopathic PH, and show a simple, direct linear relationship between *LDHA* inhibition and oxalate production. The findings also show the compound was well tolerated in these animal studies, with no adverse effects in the liver. These findings are important as they establish *LDHA* as a potential therapeutic target and open the door to a new approach to treat this family of diseases.



3. How is Dicerna's therapeutic approach different from other programs in development?

Dicerna's therapeutic approach differs from those of other companies developing therapies for PH because Dicerna targets the *LDHA* gene. Inhibition of *LDHA* was shown in animal models to reduce oxalate to normal or near-normal levels in PH types 1, 2, and ethylene glycol-induced PH. In contrast, research findings showed that inhibiting *GO* – a common target of investigational therapies for PH – does not appear to have the potential to treat PH2 or idiopathic PH.

4. What is Dicerna's timeline for initiating the DCR-PHXC clinical trial?

Dicerna is preparing to file a clinical trial application (CTA) in the EU in the fourth quarter, 2017, and plans to begin Phase 1 clinical trials in early 2018 as the Company pursues its goal of developing new therapies that address the full range of patients with PH.

5. How can I enroll in the DCR-PHXC clinical trial when it launches?

Dicerna will provide more information to the PH community about the study design, eligibility criteria, and additional details prior to starting the clinical trial.

All clinical trials have guidelines about who can participate (commonly referred to as inclusion/exclusion or eligibility criteria). Review those guidelines together with your treating physician to ensure you understand what you are signing up for before enrolling.

Once that information is available, please consult your doctor to see if this clinical trial is right for you. Also, in the near future you will find more information about DCR-PHXC and the clinical trial on clinicaltrials.gov.

6. What other non-Dicerna trials for PH are ongoing in the US and EU? Where can I learn more information about upcoming clinical trials in PH?

For the latest information about ongoing and upcoming clinical trials related to PH, check with the OHF at www.ohf.org or visit www.clinicaltrials.gov.