**Dicerna Expands Lead GalXC™ Development Program to Encompass All Forms of Primary Hyperoxaluria (PH) and Reveals New Therapeutic Target for DCR-PHXC**

*DCR-PHXC Achieved Broad, Durable, and Consistent Knockdown of LDHA and Reduction of Oxalate in Multiple Animal Models of PH*

*New Data Presented at 12th International PH Workshop Also Include Update from PHYOS Trial of Patients with PH1*

*Management to Host Conference Call Today at 10:00 a.m. ET*

**CAMBRIDGE, Mass., July 17, 2017** – Dicerna Pharmaceuticals, Inc. (NASDAQ: DRNA), a leading developer of ribonucleic acid interference (RNAi) therapeutics, presented on Saturday, July 15, 2017, new preclinical data suggesting the potential utility of DCR-PHXC, a GalXC™-based investigational therapy, for treating all forms of primary hyperoxaluria (PH). In a series of presentations at the 12th International Workshop on Primary Hyperoxaluria for Professionals, Patients and Families in Tenerife, Spain from July 14-16, 2017, Dicerna scientists presented research from animal models demonstrating 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, a group of severe, rare, inherited disorders of the liver that often result in kidney failure.

"These research findings are particularly exciting as they show that inhibition of *LDHA* reduces oxalate production in animal models of all forms of primary hyperoxaluria, establishing *LDHA* as a potentially ideal therapeutic target and opening the door to a new treatment approach for this family of diseases," noted Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "The DCR-PHXC data demonstrate potent, durable, and precise knockdown of *LDHA* in animal models of PH1, PH2 and idiopathic PH (IPH), 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. Formal animal toxicology studies are ongoing. We are preparing to file a clinical trial application in the EU in the fourth quarter, 2017, and plan to begin Phase 1 clinical trials in early 2018 as we pursue our goal of developing new therapies that address the full range of patients with PH."

In patients with PH, the liver over-produces oxalate, a metabolite that can accumulate throughout the body and particularly in the kidneys, often resulting in end-stage renal disease (ESRD) and the need for both kidney and liver transplants. DCR-PHXC, the lead investigational product candidate in Dicerna's pipeline of therapies targeting rare diseases of the liver, yields potent, liver-specific *LDHA* inhibition in animal models of PH, an effect that reduces oxalate to near-normal levels, which may prevent the damage caused to kidneys and other organs by oxalate accumulation.

At the 12th International Workshop, Dicerna presented research findings on *LDHA* inhibition, which 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 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 IPH. In animal models of PH2 and ethylene glycol-induced PH, *LDHA* knockdown with DCR-PHXC nearly normalized oxalate levels within one-month post-treatment, whereas oxalate levels remained unchanged and elevated in animals receiving GO-targeted therapy.

*LDHA* reduction has a linear correlation with oxalate reduction and offers a minimal metabolic intervention, unlike GO reduction. These benefits of *LDHA* inhibition may translate into consistent therapeutic activity even in the event of a missed dose. There are numerous case reports of *LDHA* deficiency naturally occurring in humans, with no reported adverse effects due to deficiency in the liver.

"Our data suggest that targeting *LDHA* results in thorough suppression of oxalate production," commented Bob D. Brown, Ph.D., chief scientific officer and senior vice president at Dicerna. "By reducing *LDHA* activity in the liver in animal models of PH, DCR-PHXC silences what appears to be the only pathway that is a significant producer of oxalate in all forms of primary hyperoxaluria, and seems to do so without inducing any significant liver adverse effects. These findings are very encouraging and open up a range of therapeutic opportunities for us to pursue in primary hyperoxaluria."

DCR-PHXC is the most advanced product candidate utilizing Dicerna's GalXC technology, a proprietary platform that advances the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. GalXC compounds are intended to be broadly applicable across multiple therapeutic areas, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. GalXC enables subcutaneous delivery of RNAi therapies to hepatocytes in the liver and offers several distinct potential benefits, as extensively demonstrated in various animal models. Such benefits could include potent silencing of *LDHA* and other genes; highly specific targeting to hepatocytes, sparing other cell types in the body; a long duration of action; and a simple, infrequent dosing regimen.

The 12th International Workshop also featured an update from the **P**rimary **HY**peroxaluria **O**bservational **S**tudy (PHYOS), an international, multicenter study in patients with PH1 that is collecting data on key biochemical parameters implicated in the pathogenesis of PH1 to better understand the baseline disease state, knowledge that will help guide long-term drug development plans. The study's primary objective is to measure changes in oxalate, glycolate, and other metabolites over a six-month period in patients with PH1. In a poster presentation, PHYOS investigators reported data from 20 enrolled patients with a median age at screening of 21 years (range 12-61 years). The patients had been diagnosed at a median age of 7 years (range 1-59 years), and 14 patients (74%) had a medical history of renal stones. Over the six-month observation period the variability (coefficient of variation) between 24-hour urine measurements of oxalate at different time points was 28%. These data will help the Dicerna clinical team design a clinical study using 24-hour urinary oxalate excretion as a surrogate marker for clinical benefit.

"The primary hyperoxalurias are characterized by significant unmet medical need, as there are no approved treatment options for patients living with any of these devastating diseases," said Bernd Hoppe, M.D., Head of the Division of Pediatric Nephrology in the Department of Pediatrics at the University of Bonn, Germany. "As we learn more about the natural history of primary hyperoxaluria, and as research continues to yield insights into potential therapeutic solutions, we hope to be able to offer patients new treatment options that will address the underlying cause of these debilitating diseases, and provide an alternative to combined transplantation of the kidney and liver, a highly invasive and burdensome procedure. We look forward to further updates from Dicerna and other research initiatives."

To learn more about PH, the unmet need for a treatment option and Dicerna's recent update on DCR-PHXC and its new therapeutic target, here is a link to an explanatory video.

**Conference Call Details**

Management will host a conference call at 10:00 a.m. ET on Monday, July 17, to discuss the data that were presented at the 12th International Workshop on Primary Hyperoxaluria. The conference call will be webcast live via the Internet and will be available on the "Investors & Media" section of the Dicerna website, www.dicerna.com. The webcast will also be archived on the Company's website.

The call can also be accessed by dialing (855) 453-3834 or (484) 756-4306 (international), and referencing conference ID 54633420 prior to the start of the call. After the conference call, a replay will be available until July 24, 2017. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 54633420.

**About Primary Hyperoxaluria (PH)**

Primary hyperoxaluria (PH) is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate, a natural chemical in the body that is normally eliminated as waste through the kidneys. In patients with PH, the kidneys are unable to eliminate the large amount of oxalate that is produced, and the accumulation of oxalate can result in severe damage to the kidneys and other organs. Currently, there are no approved therapies for the treatment of PH in the US.

There are three known types of PH, each of which results from a mutation in a specific gene, as well as PH for which the molecular basis remains unknown, often referred to as idiopathic PH (IPH) or "no mutation detected" (NMD) PH. The known PH mutations cause a decrease in the activity of a specific enzyme in the liver, triggering an increase in oxalate production. In each case the decreased enzyme activity changes the balance of intermediary metabolites, resulting in overproduction of oxalate. The three genetically known types of PH are: [1,2]

- PH1, which is caused by a mutation in the *AGXT* gene, causing a deficiency of the enzyme alanine:glyoxylate-aminotransferase (AGT)

- PH2, which is caused by a mutation in the *GRHPR* gene, causing a deficiency of the enzyme glyoxylate/hydroxypyruvate reductase (GR/HPR)
- PH3, which is caused by a mutation in the *HOGA1* gene, causing a deficiency of the enzyme 4-hydroxy-2-oxoglutarate aldolase (HOGA)

Patients with PH often undergo both liver and kidney transplants, which are major surgical procedures, and subsequently must take immunosuppressant drugs for the rest of their lives. Patients with decreased renal function may also experience oxalosis, which involves a build-up of oxalate in other organs such as the bone, skin, heart, and retina, possibly causing other concomitant, debilitating complications.

The estimated genetic incidence of PH1, the most common type of PH, is 1 in 151,887 births, which implies more than 5,000 patients in the US and EU have the disease.[3] The median age at the first appearance of PH1 symptoms is 5.8 years.[4] The median age at diagnosis of PH1 is between 4.2 and 11.5 years, depending on whether nephrocalcinosis (calcification in the renal parenchyma, the functional part of the kidney) is present.[5] Fifty percent of patients with PH1 reach end-stage renal disease (ESRD) by their mid-30s.[2]

**About Dicerna Pharmaceuticals, Inc.**

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative RNAi-based therapeutics for diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company is leveraging its proprietary GalXC™ RNAi technology platform to build a broad pipeline in these core therapeutic areas, focusing on target genes where connections between target gene and diseases are well understood and documented. The Company intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. For more information, please visit www.dicerna.com.

**Cautionary Note on Forward-Looking Statements**

This press release includes forward-looking statements. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. GalXC-mediated delivery technology and pipeline programs, including DCR-PHXC, are in preclinical development, and the process by which a preclinical technology and product candidates could potentially lead to an approved product is long and subject to significant risks and uncertainties. Applicable risks and uncertainties include those relating to our preclinical and clinical research and other risks identified under the heading "Risk Factors" included in our most recent Form 10-K filing and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Dicerna's current views with respect to future events, and Dicerna does not undertake and specifically disclaims any obligation to update any forward-looking statements.

**References**

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