Dicerna Announces Data to Be Presented at American Society of Nephrology (ASN) Kidney Week 2021

October 15, 2021

– Data From PHYOX™2 Pivotal Trial of Nedosiran for Treatment of Primary Hyperoxaluria (PH) Accepted as Late-Breaker Poster Presentation –

– Company on Track to Submit Nedosiran New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for Treatment of PH Type 1 (PH1) in Fourth Quarter of 2021 –

– Dicerna Continues to Evaluate PH2 Outcomes in Consultation With Scientific Advisors, Clinicians and the Patient Community –

LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 15, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced an abstract on clinical data from the Company's PHYOX™2 trial of nedosiran, an investigational GalXC™ RNAi candidate for the treatment of primary hyperoxaluria (PH), has been accepted as a late-breaker poster presentation at the American Society of Nephrology (ASN) Kidney Week 2021 taking place Nov. 4-7, 2021.

"PHYOX2 generated robust data in patients with PH1, meeting the primary and key secondary efficacy endpoints, while also showing that nedosiran was safe and generally well tolerated in the trial," said Douglas Fambrough, Ph.D., President and Chief Executive Officer at Dicerna. "We are excited to share these results and believe that the selection of nedosiran data for a late-breaker presentation at ASN Kidney Week underscores the importance of providing the medical community with new information about potential therapy options for the treatment of patients with PH1. We continue to believe that these data support potential approval of nedosiran for the treatment of PH1 in the U.S., Europe, Japan and other markets, and we remain on track to submit an NDA to the FDA in the fourth quarter."

Dr. Fambrough continued, "We continue to work in consultation with scientific advisors, clinicians and the patient community to evaluate the outcomes observed in patients with PH type 2 (PH2). Given the significant unmet medical need, we believe further assessment of nedosiran's potential in treating PH2 is warranted. We are actively engaged in out-licensing discussions to commercialize nedosiran for patients with PH1 and have expanded these interactions to include the potential for continued research and clinical development in PH2."

The schedule for Dicerna's presentations is as follows:

Session: Late-Breaking Clinical Trial Posters
Poster Title: *PHYOX™2: Nedosiran Reduced Urinary Oxalate Excretion in Patients With Primary Hyperoxaluria*
Poster #: PO2538

A poster summarizing an analysis of healthcare utilization and outcomes in patients with PH and an informational poster on additional trials in the PHYOX clinical development program will also be presented.

Session: Genetic Diseases of the Kidneys: Non-Cystic – 1
Poster Title: *Real-World Healthcare Utilization and Clinical Markers Preceding Dialysis in Patients with Primary Hyperoxaluria (PH) in the United States*
Poster #: PO1317

Session: Informational Posters
Poster Title: *The PHYOX™Clinical Program for Primary Hyperoxaluria*
Poster #: INFO22

All three posters will be available on the ASN website starting Thursday, Nov. 4 at 10:00 a.m. PDT/1:00 p.m. EDT.

About Primary Hyperoxaluria (PH)

Primary hyperoxaluria (PH) is a family of ultra-rare, life-threatening genetic disorders that initially manifest with complications in the kidneys. There are three known subtypes of PH (PH1, PH2 and PH3), each resulting from a mutation in one of three different genes. These genetic mutations cause enzyme deficiencies that result in the overproduction of oxalate, which is an end-product of metabolism. Excess production and accumulation of oxalate leads to recurrent kidney stones, nephrocalcinosis and chronic kidney disease that may progress to end-stage renal disease requiring intensive dialysis. Compromised renal function eventually results in the accumulation of oxalate in a wide range of organs including the skin, bones, eyes and heart. In the most severe cases, symptoms start in the first year of life. A combined liver-kidney transplant may be undertaken to resolve PH1 or PH2, but it is an invasive solution with limited availability and high morbidity that requires lifelong immune suppression to prevent organ rejection. Genetic studies suggest approximately 8,500 people in the U.S. are affected by PH, and researchers estimate that more than 80% of patients remain undiagnosed.[1] There is currently only one approved therapy available that is limited to the treatment of patients with PH1.

About Nedosiran

Nedosiran is in development for the treatment of primary hyperoxaluria (PH) as part of the PHYOX™ clinical development program and is Dicerna's most advanced RNAi drug candidate utilizing our proprietary GalXC™ RNAi technology. Nedosiran is designed to inhibit production of the hepatic lactate dehydrogenase (LDH) enzyme – an enzyme that catalyzes the final step in the glyoxylate metabolism pathway that can lead to oxalate overproduction in patients with PH.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) is a biopharmaceutical company focused on discovering, developing and commercializing medicines that are designed to leverage ribonucleic acid interference (RNAi) to silence selectively genes that cause or contribute to disease. Using our proprietary GalXC™ and GalXC-Plus™ RNAi technologies, Dicerna is committed to developing RNAi-based therapies with the potential to treat both rare and more prevalent diseases. By silencing disease-causing genes, Dicerna's GalXC platform has the potential to address conditions that are difficult to treat with other modalities. Initially focused on disease-causing genes in the liver, Dicerna has continued to innovate and is exploring new applications of its RNAi technology with GalXC-Plus, which expands the functionality and application of our flagship liver-targeted GalXC technology to tissues and cell types outside the liver, and has the potential to treat diseases across multiple therapeutic areas. In addition to our own pipeline of core discovery and clinical candidates, Dicerna has established collaborative relationships with some of the world's leading pharmaceutical companies, including Novo Nordisk A/S, Roche, Eli Lilly and Company, Alexion Pharmaceuticals, Inc., Boehringer Ingelheim International GmbH and Alnylam Pharmaceuticals, Inc. Between Dicerna and our collaborative partners, we currently have more than 20 active discovery, preclinical or clinical programs focused on cardiometabolic, viral, chronic liver and complement-mediated diseases, as well as neurodegenerative diseases and pain. At Dicerna, our mission is to interfere – to silence genes, to fight disease, to restore health. For more information, visit www.dicerna.com.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements including (a) the expected timing of submitting our NDA for nedosiran to the FDA for the treatment of PH1, our belief that data from PHYOX2 in patients with PH1 support potential approval of nedosiran for the treatment of PH1 in the U.S., Europe, Japan and other markets, and our belief that further assessment of nedosiran's potential in treating PH2 is warranted; as well as (b) such statements pertaining to the Company's planned participation at a scientific conference, which may include discussion of the Company's business and operations, including the discovery, development and commercialization of our product candidates and technologies, and the therapeutic potential thereof, the success of our collaborations with partners and any potential future collaborations. 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. Applicable risks and uncertainties include those relating to our preclinical research and clinical programs and other risks identified under the heading "Risk Factors" included in our most recent Form 10-Q and Form 10-K filings 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.

[1] Hopp K, et al. J Am Soc Nephrol. 2015;26(10):2559-2570 and U.S. Census Bureau population on a date: February 20, 2020. United States Census Bureau website, 2020.

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