Dicerna to Present Nedosiran Data at American Society of Nephrology (ASN) Kidney Week 2020

October 9, 2020

LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 9, 2020-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the “Company” or “Dicerna”), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced that clinical data on nedosiran, an investigational candidate for the treatment of primary hyperoxaluria (PH), will be presented during the American Society of Nephrology (ASN) Kidney Week taking place Oct. 22-25, 2020. In addition, a commercial claims analysis of clinical and economic burden associated with PH will also be presented.

The following poster presentations will be available during the “Genetic Diseases of the Kidneys: Non-Cystic – 1” session taking place on Thursday, Oct. 22, 2020 at 10:00 a.m. ET:

- **Title:** PHYOX™3: A Long-Term, Open-Label Extension Trial of Nedosiran in Patients With Primary Hyperoxaluria Type 1, 2 or 3
  Poster #: PO1625

- **Title:** Clinical and Economic Impact of Primary Hyperoxaluria: A Retrospective Claims Analysis
  Poster #: PO1627

About Primary Hyperoxaluria

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 types 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 a substrate called oxalate. Abnormal 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 organs ranging from skin, bones, eyes and heart. In the most severe cases, symptoms start in the first year of life. A combined liver-kidney transplantation 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. There are currently no approved medications for the treatment of PH. Patients are limited to using hyperhydration and medication to attempt to increase solubility of oxalate in urine. Despite these interventions, oxalate may continue to accumulate in the kidneys and other organs, causing damage.

About Nedosiran

Nedosiran is the only RNAi drug candidate in development for primary hyperoxaluria (PH) types 1, 2 and 3 and is Dicerna’s most advanced product candidate utilizing the proprietary GalXC™ RNAi technology platform. Nedosiran is designed to reduce the levels of hepatic lactate dehydrogenase (LDH) enzyme – an enzyme that catalyzes the final step in a common pathway resulting in oxalate overproduction in patients with PH1, PH2 and PH3. Dicerna is evaluating the safety and efficacy of nedosiran in patients with all known forms of PH as part of its PHYOX™ clinical development program.

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 selectively silence genes that cause or contribute to disease. Using our proprietary RNAi technology platform called GalXC™, 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 hepatocytes, Dicerna has continued to innovate and is exploring new applications of its RNAi technology beyond the liver, targeting additional tissues and enabling new therapeutic applications. 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 rare, cardiometabolic, viral, chronic liver and complement-mediated diseases, as well as neurodegeneration and pain. At Dicerna, our mission is to interfere – to silence genes, to fight disease, to restore health. For more information, please visit www.dicerna.com.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements pertaining to the Company’s planned attendance and presentation at a virtual scientific conference, which will include discussion of the Company’s clinical results related to nedosiran and an insurance claims analysis for primary hyperoxaluria, as well as to our business and operations, including the discovery, development and commercialization of our product candidates and technology platform, and the therapeutic potential thereof, the success of our collaboration 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 risks identified under the heading “Risk Factors” included in our most recent Form 10-Q 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.

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