Dicerna Announces Enrollment Completion of PHYOX™2 Pivotal Trial of Nedosiran for Treatment of Primary Hyperoxaluria

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– Dicerna Expects Trial Completion in First Half of 2021, With Top-Line Data Available Mid-Year –

– Enrollment in PHYOX4 Trial for PH3 Expected to Begin in January 2021 –

– Planned New Drug Application Submission On Track for Third Quarter of 2021 –

LEXINGTON, Mass.--(BUSINESS WIRE)--Jan. 4, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced enrollment completion in the Company's PHYOX™2 pivotal, double-blind, randomized, placebo-controlled clinical trial of its lead investigational therapy, nedosiran, which is in development as a once-monthly treatment of all three known types of primary hyperoxaluria (PH) – PH1, PH2 and PH3. PH is a family of ultra-rare, life-threatening genetic disorders that initially manifest with complications in the kidneys. The Company expects the last patient visit in the PHYOX2 trial to occur in the first half of 2021 and to report top-line results from the study mid-year.

"This milestone reflects the significant progress we've made in the PHYOX clinical trial program this year in spite of the challenges presented by the COVID-19 pandemic. We are grateful to all of our collaborating investigators and participants for their commitment. With enrollment in our pivotal study of nedosiran for PH now completed, we are one step closer to potentially providing a convenient and differentiated treatment option that addresses the underlying cause of disease for patients with PH1, PH2 and PH3," said Shreeram Aradhye, M.D., Executive Vice President and Chief Medical Officer at Dicerna. "We look forward to sharing top-line results from PHYOX2 later this year, with the goal of submitting a New Drug Application for nedosiran in the third quarter of 2021."

The PHYOX2 multicenter pivotal trial (NCT03847909) is designed to evaluate the efficacy and safety of nedosiran delivered as a once-monthly subcutaneous injection in participants aged six years and older who have PH1 or PH2. The primary endpoint of the study is the percent change from baseline in 24-hour urinary oxalate excretion between Days 90 and 180. Enrollment of 35 participants was completed in December 2020 and included participants across 11 countries, including the U.S., Japan and Europe.

PHYOX2 is part of the broader PHYOX clinical trial program designed to evaluate nedosiran in PH1, PH2 and PH3 patients of all ages and stages of chronic kidney disease. The PHYOX4 trial in patients with PH3 is now expected to begin enrollment in January 2021. Data from PHYOX1, PHYOX2, PHYOX4, the ongoing PHYOX3 open-label extension study, and the PHYOX-OBX natural history study of PH3 participants, are expected to form the basis of the nedosiran New Drug Application (NDA) submission. The Company anticipates initiating supplemental studies, including PHYOX7 in patients with end-stage renal disease, and PHYOX8 in pediatric patients younger than six years of age, in the first and second quarters of 2021, respectively.

Participants who complete PHYOX trials are eligible to enroll in the Company's PHYOX3 trial, an ongoing open-label extension study evaluating nedosiran's long-term safety and efficacy. An interim analysis of the PHYOX3 trial presented at the American Society of Nephrology's Kidney Week 2020 annual scientific conference in October 2020 showed all participants receiving nedosiran, regardless of PH1 or PH2 subtype, achieved normalization or near-normalization of urinary oxalate (Uox), a key PH measure, by Day 180. Of the 13 participants who reached Day 180 as of the interim analysis, 10 (100%) of the participants with PH1, and two of the three (67%) participants with PH2, achieved normal Uox excretions at one or more visits, and 62% of all participants achieved normal Uox excretions on at least three consecutive visits, meeting protocol-defined eligibility for gradual reduction in fluid intake requirements.

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 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 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 specifically for PH that is limited to the treatment of patients with PH1.

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 inhibit production of the hepatic lactate dehydrogenase (LDH) enzyme – an enzyme that catalyzes the final step in the glyoxalate metabolism pathway that can lead to oxalate overproduction in patients with PH1, PH2 or 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 silence selectively 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. 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. Examples of forward-looking statements include, among others, statements we make regarding our product candidates and the development thereof, including the progress of the Company's PHYOX2 and other trials of nedosiran, results from future trials of the Company's PHYOX clinical development program, the therapeutic potential of our product candidates, including nedosiran, the planned submission of the New Drug Application for nedosiran, 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, our collaboration with partners and any potential future collaborations. The process by which investigational therapies, such as nedosiran, could potentially lead to an approved product is long and subject to highly significant risks. Applicable risks and uncertainties include those relating to Dicerna's clinical research and other risks identified under the heading "Risk Factors" included in the Company's most recent filings on Forms 10-K and 10-Q and in other future filings with the Securities and Exchange Commission. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by us and our collaborative partners; the likelihood of Dicerna's clinical programs being executed on timelines provided and reliance on the Company's contract research organizations and predictability of timely enrollment of subjects and patients to advance Dicerna's clinical trials; the reliance of Dicerna on contract manufacturers to supply its products for research, development and commercialization and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the impact of the ongoing COVID-19 pandemic on our business operations, including the conduct of our research and development activities; the regulatory review and unpredictability of the duration and results of the regulatory review of Investigational New Drug applications (INDs) and Clinical Trial Applications (CTAs) that are necessary to continue to advance and progress the Company's clinical programs; the timing, plans and reviews by regulatory authorities of marketing applications such as New Drug Applications (NDAs) and comparable foreign applications for one or more of Dicerna's product candidates; continued alignment with the FDA on the regulatory pathway to approval for nedosiran; the ability to secure, maintain and realize the intended benefits of collaborations with partners; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in R&D and following commercialization; and general business, financial, and accounting risks and litigation. 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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