Dicerna Announces Dosing Completion in Nedosiran PHYOX™4 Clinical Trial for Treatment of Primary Hyperoxaluria Type 3

June 16, 2021

– Company Continues to Expect Nedosiran New Drug Application Submission in Fourth Quarter of 2021 –

LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 16, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced it has completed dosing in the Company's PHYOX™4 single-dose safety and tolerability study of its investigational candidate, nedosiran, for the treatment of primary hyperoxaluria (PH) type 3 (PH3). Nedosiran is Dicerna's lead investigational GalXC™ RNAi therapy and is in development as a once-monthly subcutaneous treatment for all three known types of PH (PH1, PH2 and PH3), a family of ultra-rare, life-threatening genetic disorders that initially manifest with complications in the kidneys. The Company expects to report top-line results from this study in October 2021.

"Meaningful progress has been made in the last two years to better understand the burden of disease for patients with PH," said Shreeram Aradhye, M.D., Executive Vice President and Chief Medical Officer at Dicerna. "With increasing evidence suggesting people living with PH3 suffer from consequences as serious as those with PH1 and PH2,[1] including potential progression to end-stage renal disease, there is a significant need to provide a new therapeutic option that can treat all patients, including those with PH3. The nedosiran PHYOX4 trial is the first clinical trial of any investigational therapy in patients with PH3, and we're gratified to be at the forefront in advancing the state of the science for PH."

The PHYOX4 trial (NCT04555486) is a randomized, placebo-controlled, double-blind, multicenter study designed to evaluate the safety and tolerability of a single subcutaneous dose of nedosiran in six patients with PH3 who have had at least one kidney stone event in the last 12 months. The study will also assess the proportion of participants achieving more than a 30% decrease from baseline in 24-hour urinary oxalate (Uox) on two consecutive visits. PHYOX4 participants who respond to treatment with nedosiran and complete the trial are also eligible to enroll in the Company's PHYOX3 open-label extension study evaluating nedosiran's long-term safety and efficacy in participants with PH1, PH2 or PH3.

PHYOX4 is part of the broader PHYOX clinical trial program designed to evaluate nedosiran in patients with PH1, PH2 and PH3. Data from PHYOX1, a single-dose Phase 1 trial in healthy volunteers and patients with PH1 or PH2; PHYOX2, the pivotal, double-blind, placebo-controlled, six-month trial in patients with PH1 or PH2; PHYOX4; and the ongoing PHYOX3 study are expected to support the nedosiran New Drug Application (NDA) submission, which is planned for the fourth quarter of 2021.

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.[2] 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 glyoxylate 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 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 on the functionality and application of our flagship liver-targeted GalXC technology, 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, 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 PHYOX4 trial 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 (NDA) 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 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] Martin-Higueras, et al. Systemic Oxalosis in Primary Hyperoxaluria Type 3 – Are the Patients at Risk? Nephrology Dialysis Transplantation 36 (Supplement 1): i142–i146, 2021 10.1093/ndt/qfab107. Mini-Oral Presentation at ERA-EDTA 2021 Congress.

[2] 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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