



## Dicerna Provides Initial Observations From PHYOX™3 Trial of Nedosiran for Treatment of Primary Hyperoxaluria and an Update on Data Presentation Plan

March 31, 2020

— Preliminary Interim Data Show Normalization or Near-Normalization of Oxalate Levels in First Subset of Four Patients —

— Nedosiran Multidose Data to Be Presented at OxalEurope Meeting Now Rescheduled to June 16, 2020 —

LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 31, 2020-- [Dicerna Pharmaceuticals, Inc.](#) (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of ribonucleic acid interference (RNAi) therapies, today provided an overview of initial observations from the PHYOX™3 long-term, multidose, open-label rollover extension trial evaluating nedosiran, the Company's lead product candidate in development for primary hyperoxaluria (PH) types 1, 2 and 3, and provided an update on its data presentation plan.

In March 2020, Dicerna conducted a preliminary analysis of multidose data from the PHYOX3 trial that the Company planned to present at the OxalEurope (The European Hyperoxaluria Consortium) Meeting, which has now been postponed to June 2020. As of this preliminary data analysis, 14 participants from the completed single-dose PHYOX1 Phase 1 clinical trial had enrolled in the PHYOX3 trial, and four had received at least three monthly doses of nedosiran delivered subcutaneously. In this preliminary analysis, nedosiran appeared well tolerated, with no injection-site reactions (defined as occurring four hours or more after injection) and no drug-related severe adverse events. The overall adverse event profile was comparable to that observed in the PHYOX1 Phase 1 clinical trial. There were two patients who each experienced a serious adverse event, but these were determined by the investigator to be unrelated to the study drug. Based on the cumulative number of days patients have participated in the PHYOX3 trial, total patient exposure to monthly dosing of nedosiran delivered subcutaneously has reached nearly two years. The longest-treated patient in the PHYOX3 trial has now received seven monthly doses of nedosiran.

"We are encouraged by the initial data emerging from our multidose PHYOX3 open-label trial, which is designed to evaluate nedosiran's effect on renal function and assess its safety and long-term effect in reducing urinary oxalate levels with chronic monthly dosing in patients with PH," said Ralf Rosskamp, M.D., chief medical officer at Dicerna. "The PHYOX3 trial continues to progress according to plan, with data from this trial showing, on at least two visits, normalization or near-normalization of urinary oxalate levels for four patients who have received at least three monthly doses of nedosiran, and a favorable tolerability profile. We look forward to the opportunity to present a more advanced data set at this scientific forum in June, as more patients receive multiple doses of nedosiran."

The PHYOX3 trial ([ClinicalTrials.gov](#): NCT04042402) is designed to evaluate nedosiran's long-term safety and efficacy in patients with PH1, PH2 or PH3. The PHYOX3 trial is an open-label extension study for patients six years of age or older with PH who have participated in any previous PHYOX clinical development program trial. Normal and near-normal urinary oxalate levels in the PHYOX3 trial are defined as below 0.46 mmol and from 0.46 to 0.6 mmol, respectively, during a 24-hour period.

The OxalEurope Meeting, previously scheduled for March 31, 2020, is now expected to take place as either a virtual or in-person meeting in Amsterdam on June 16, 2020. Dicerna plans to present the PHYOX3 multidose data at the rescheduled event.

### 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 results eventually in the accumulation of oxalate in organs ranging from skin, bones, eyes and heart, especially in patients with PH1. 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 is an invasive solution with limited availability and high morbidity that requires lifelong immune suppression to prevent organ rejection. Currently, there is no approved therapy 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, causing damage.

### About Nedosiran

Nedosiran (formerly referred to as DCR-PHXC) 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 the 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, GalXC™, Dicerna is committed to developing RNAi-based therapies with the potential to treat both rare and more prevalent diseases. By reducing the level of disease-causing proteins in the hepatocytes of the liver, Dicerna's GalXC platform has the potential to safely target conditions that are difficult to treat with other modalities. Continually innovating, Dicerna is also exploring new applications of 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. and Boehringer Ingelheim International GmbH. Between Dicerna and

our collaborative partners, we currently have more than 20 active discovery, preclinical or clinical programs focused on rare, cardiometabolic, viral-infectious, 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](http://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: (i) the therapeutic and commercial potential of nedosiran and the GalXC™ platform; (ii) research and development plans and timelines related to nedosiran, including additional patients receiving multiple doses of nedosiran, and the availability of more advanced data for presentation at the rescheduled OxalEurope Meeting in June 2020; (iii) the timing of the rescheduled OxalEurope Meeting and Dicerna's presentation of data as part of the meeting; and (iv) continued enrollment and progress of the PHYOX3 trial. 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 filing on Forms 10-K and in other future filings with the Securities and Exchange Commission. These risks and uncertainties include, among others, future conduct of the business of the Company, its clinical programs and operations in the face of the COVID-19 pandemic; 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 and development 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 unpredictability of the duration and results of the regulatory review of Investigational New Drug applications (INDs) and Clinical Trial Applications that are necessary to continue to advance and progress the Company's clinical programs and the regulatory review of marketing applications in the future; 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 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.

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#### **Media:**

Amy Trevvett, Dicerna Pharmaceuticals, Inc.  
+1 617-612-6253  
[atrevvett@dicerna.com](mailto:atrevvett@dicerna.com)

#### **Investors:**

Lauren Stival, Stern Investor Relations, Inc.  
+1 212-362-1200  
[lauren.stival@sternir.com](mailto:lauren.stival@sternir.com)

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