



## Dicerna Addresses Business Continuity in Response to COVID-19 Pandemic

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LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 26, 2020-- [Dicerna Pharmaceuticals, Inc.](#) (Nasdaq:DRNA) (the "Company" or "Dicerna"), a leading developer of ribonucleic acid interference (RNAi) therapies, today provided an overview of its response to the COVID-19 pandemic and an update on business continuity and clinical development milestones. Dicerna has been closely monitoring the rapidly evolving situation with the COVID-19 pandemic and has taken measures to help ensure the health of its employees, participants in ongoing clinical studies, families and caregivers, and patient communities.

"The COVID-19 crisis reminds us that the health and safety of our people, clinical trial participants and our communities is our first priority. Like many other companies in drug development, a number of our clinical trial sites are temporarily postponing or suspending trial-related activities in the wake of COVID-19, impacting our clinical trial execution plans," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "Today, we are providing an update on our business and key trials within our core programs that reflect the recent clinical developments and our expectations based on currently available information. We will continue to monitor each of the ongoing trials across our development programs with the goal of ensuring trial continuity and advancement where feasible. While we are operating in what can only be described as a highly fluid situation, the fundamentals of our business remain strong, and we expect to provide data on our clinical programs at our R&D Day in early August as previously planned."

### Patients and Clinical Trials

Dicerna conducts clinical trials in various countries around the world, including the U.S. and other areas heavily impacted by the pandemic, and is closely monitoring the daily updates from regulatory agencies, such as the U.S. Food and Drug Administration and European Medicines Agency, and implementing their guidance. Governments in each of the territories in which Dicerna operates are evaluating the impact and appropriate response to contain the spread of the SARS-CoV-2 virus, including limitations on business operations and non-essential travel, as well as restrictions on interpersonal contact and environments that are a higher risk for virus transmission. Similarly, health care institutions and clinical trial sites are implementing restrictive measures to reduce participants' and employees' potential exposure to the virus. As a result, and based on the most recent updates from clinical sites impacted by these measures, the Company has reevaluated its previous expectations related to clinical development milestones as follows:

- **PHYOX™2 Pivotal Trial of Nedosiran for Primary Hyperoxaluria (PH).** Dicerna is instituting measures to ensure continuity of care and continued dosing of patients currently enrolled in the PHYOX2 trial. The Company is working closely with local Institutional Review Boards (IRBs) to implement a protocol amendment that would allow for the transition of remaining site visits to at-home nurse visits for drug administration and safety follow-up. Based on current enrollment and temporary suspension of further clinical trial activities at multiple sites, the Company no longer expects to complete enrollment in PHYOX2 in the second quarter of 2020 as previously projected. Given the fluid nature of the current situation, and the evolving and extraordinary actions undertaken by clinical trial sites globally, the Company is reevaluating the clinical plan, and at a later date, will provide a revised timing estimate for PHYOX2 enrollment completion, and evaluate its potential effect on timing of subsequent activities, such as the nedosiran New Drug Application submission.
- **PHYOX3 Long-Term Multidose Trial of Nedosiran for PH.** Unlike the PHYOX2 trial, which requires screening and enrollment of new patients, patients with PH are permitted to roll over into the PHYOX3 trial from any previous nedosiran trial in which they have participated. As with PHYOX2, Dicerna is working with local IRBs to implement a protocol amendment to facilitate transition of remaining site visits to home-based dose administration and safety follow-up and, at this time, expects that a modified protocol would enable the PHYOX3 trial to continue according to plan for most participants.
- **RG6346 Phase 1 Proof-of-Concept Trial for Hepatitis B Virus Infection.** The Phase 1 clinical trial of RG6346 for the treatment of chronic hepatitis B virus (HBV) infection continues to progress as planned and is nearing the end of enrollment. Dicerna continues to expect to present Phase 1 proof-of-concept data from all existing cohorts at the Company's R&D Day in August 2020.
- **DCR-A1AT Phase 1/2 Trial for Alpha-1 Antitrypsin Deficiency (A1AT).** The initial enrollment of healthy volunteers has proceeded according to plan in Dicerna's Phase 1/2 trial of DCR-A1AT. However, given the potential for increased respiratory risk from COVID-19 for future participants in this trial, the Company currently expects that a pause in enrollment at higher doses in the Phase 1/2 trial is likely, pending further developments in management of the COVID-19 situation.
- **R&D Day.** Dicerna continues to plan to host an R&D Day event in August 2020, either virtually or in person, as circumstances allow, with updates on nedosiran multidose data, RG6346 Phase 1 data and new data regarding the extension of the Company's RNAi technology to additional tissues.

Due to the rapidly evolving circumstances, Dicerna is continuing to evaluate the impact to future studies of nedosiran and future activities in its other clinical trials and development programs. The Company expects to provide a further update when it announces its first quarter 2020 financial results.

## Product Supply and Supply Chain

Supply of Dicerna's investigational medicines is sufficient to support ongoing clinical trials. Based on current evaluations, Dicerna's supply chains continue to appear intact at this time to meet the Company's foreseeable 2020 clinical, nonclinical and chemistry, manufacturing and control (CMC) supply demands across all programs, but the Company is alert to the potential for disruptions that could arise from COVID-19 and remains in close contact with suppliers.

## Cash Position

Dicerna continues to be well capitalized with approximately \$720 million in unaudited cash, cash equivalents and marketable securities as of Feb. 29, 2020. The Company continues to believe that its available cash, cash equivalents and marketable securities will be sufficient to fund operations into 2023.

## Employees and Communities

Dicerna has instituted a mandatory work-from-home policy for the majority of its employees at each location to stem the spread of the COVID-19 virus and enable the continued health and safety of its work force. The duration of this remote working arrangement will be guided by the direction of the state governments in which Dicerna operates, and actions and guidelines issued by the federal government, including the Centers for Disease Control and Prevention. To facilitate an effective remote-work policy and to ensure business continuity, Dicerna has also made available to its employees an enhanced suite of technology resources to permit secure remote access and to enable business to continue virtually.

Due to the nature of Dicerna's work and mission, essential-work exemptions continue to permit critical research and development and laboratory activities for limited personnel. Those exemptions enable some continued discovery research and activities supporting the Company's collaborative agreements and Dicerna's own programs. For these employees, Dicerna has established a set of safety guidelines to reduce close interactions, including flexible work schedules to limit the number of people on-site and use of personal protective equipment, which are intended to maintain the safety of employees, their families and communities.

## 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) future conduct of the business of the Company, its clinical programs and operations in the face of the COVID-19 pandemic; (ii) the research and development plans and timelines related to the Company's clinical programs and the opportunity to enroll, continue or resume clinical studies that are slowed or halted by the COVID-19 pandemic; and (iii) continued manufacture and supply of raw materials for the Company's clinical and development programs, the availability of any of which could be significantly impaired by COVID-19. 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 Form 10-K 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 whose operations and activities may be slowed or halted by the COVID-19 pandemic; 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 and maintain their own operations; 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 and Clinical Trial Applications that are necessary to initiate and 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.

## Cautionary Note on Future Updates

The statements contained in this press release reflect Dicerna's current views with respect to future events, which may change significantly as the global consequences of the COVID-19 pandemic rapidly develop. Accordingly, Dicerna does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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