Dicerna Receives Rare Pediatric Disease Designation From U.S. Food and Drug Administration for Nedosiran for Treatment of Primary Hyperoxaluria

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LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 18, 2020-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the “Company” or “Dicerna”), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced that the U.S. Food and Drug Administration (“FDA” or the “agency”) has granted rare pediatric disease designation for nedosiran, an investigational RNAi therapy being developed as a once-monthly treatment for primary hyperoxaluria (PH). PH is a family of ultra-rare, life-threatening genetic disorders that cause 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. Nedosiran is the Company’s lead product candidate and is in development for PH types 1, 2 and 3.

Under the FDA's rare pediatric disease designation program, the FDA may grant a priority review voucher to a sponsor who receives a product approval for a “rare pediatric disease,” which is defined as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the U.S. Subject to FDA approval of nedosiran for the treatment of PH, Dicerna would be eligible to receive a voucher that may be redeemed to receive priority review for a subsequent marketing application for a different product candidate or which could be sold or transferred.

“The FDA’s rare pediatric disease designation for nedosiran for primary hyperoxaluria reflects the agency’s continued recognition that PH is a serious and potentially fatal condition, with disease consequences that frequently manifest in childhood but progress over time into adulthood,” said Ralf Rosskamp, M.D., chief medical officer at Dicerna. “These patients and their caregivers cope with burdensome and inadequate disease management techniques that ultimately cannot cure their underlying disease or prevent its potential to result in kidney stones, nephrocalcinosis, growth failure and failure to thrive, systemic oxalosis and end-stage renal disease – serious manifestations that often affect children. Our development program for nedosiran for the treatment of PH1, PH2 and PH3 reflects our recognition of the unmet need among all patients with PH, regardless of age or PH type, and we are gratified by this new designation as we continue to work toward our goal of providing a treatment for patients with all known forms of PH.”

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. 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 called 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 genes of the liver, Dicerna’s GalXC 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., 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: (i) the therapeutic and commercial potential of nedosiran and (ii) clinical development timelines and review related to nedosiran, including ultimate review of the nedosiran pediatric clinical development program, and based on such review, award of a pediatric voucher under the FDA’s pediatric rare disease program and continued alignment on the regulatory pathway to approval. 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 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 impact of the ongoing COVID-19 pandemic on our business operations, including the conduct of our research and development activities; the 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 and the regulatory review of INDs and CTAs; 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; 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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