



Dicerna Announces FDA Acceptance of Lilly's Investigational New Drug (IND) Application for LY3819469

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– Second GalXC™ RNAi Investigational New Drug (IND) Application Filed by Lilly Under Companies' Global Research Collaboration and Licensing Agreement –

LEXINGTON, Mass.--(BUSINESS WIRE)--May 27, 2021-- [Dicerna Pharmaceuticals, Inc.](#) (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced the U.S. Food and Drug Administration ("FDA") acceptance of the Investigational New Drug ("IND") application filed by [Eli Lilly and Company](#) ("Lilly") for LY3819469, the second clinical-stage candidate to emerge from Dicerna's collaboration with Lilly. The IND milestone triggers a \$10 million payment to Dicerna and enables Lilly to initiate a Phase 1 clinical trial of LY3819469, an investigational GalXC™ RNAi candidate targeting the *PLA* gene as a potential treatment of cardiometabolic diseases.

"This milestone marks the second IND generated through our productive collaboration with Lilly," said Bob D. Brown, Ph.D., Chief Scientific Officer and Executive Vice President of R&D at Dicerna. "In addition to the two candidates now at clinical stage, there are so far, nine discovery research programs connected to this alliance, emphasizing Dicerna's and Lilly's shared commitment to bringing forward new RNAi-based therapies to treat a broad range of diseases."

The IND filing for LY3819469 is the second development milestone achieved under a 2018 global licensing and research collaboration between Dicerna and Lilly focused on the discovery, development and commercialization of potential new therapies for cardiometabolic and neurodegenerative diseases and pain. This investigational cardiometabolic therapy and future therapies to emerge from the two companies' collaboration leverage Dicerna's proprietary GalXC RNAi technology platform.

Under the agreement, Dicerna is eligible to receive up to \$350 million in development and commercialization milestones for each GalXC hepatocyte target and \$355 million for each non-hepatocyte target, as well as tiered royalties ranging from the mid-single-digits to low double-digits on potential product sales.

About RNAi and Dicerna's GalXC™ RNAi Platform Technologies

Ribonucleic acid interference, or RNAi, provides a unique advantage to other disease inhibitor technologies, like small-molecule pharmaceuticals or monoclonal antibodies. Instead of targeting proteins after they have been produced and released, RNAi silences the genes themselves via the specific destruction of the messenger RNA (mRNA) made from the gene. Rather than seeking to inhibit a protein, the RNAi approach can prevent a disease-causing protein's creation, directly impacting disease manifestation.

Dicerna's proprietary GalXC™ RNAi platform aims to advance the development of next-generation RNAi-based therapies. Investigational therapeutics developed using our flagship GalXC technology utilize a proprietary *N*-acetyl-D-galactosamine (GalNAc)-mediated structure of double-stranded RNA molecules that are designed to bind specifically to receptors on liver cells, leading to selective hepatocyte internalization and access to the RNAi machinery within the cells. Dicerna is continuously innovating and exploring new applications of RNAi technology beyond GalNAc-mediated delivery to the liver, including alternative RNA structures and fully synthetic ligands that target other tissues and enable new therapeutic applications, referred to as GalXC-Plus™.

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 the collaboration agreement with Lilly and the therapeutic potential of LY3819469 and other development candidates under the agreement with Lilly. The process by which investigational therapies 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 (IND) applications 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.

GalXC™ and GalXC-Plus™ are trademarks of Dicerna Pharmaceuticals, Inc.

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