Dicerna Announces FDA Acceptance of Lilly's Investigational New Drug (IND) Application for First GalXC™ RNAi Candidate Under Companies' Global Research Collaboration and Licensing Agreement

November 16, 2020

— Milestone Achievement Reflects First Investigational Candidate to Emerge Under Multi-Target Collaboration and Triggers $10 Million Payment to Dicerna —

– Lilly Targeting Initiation of Phase 1 Clinical Trial of LY3561774 by Year-End 2020 –

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 16, 2020-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA), (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference ("RNAi") therapeutics, today announced U.S. Food and Drug Administration ("FDA") acceptance of the Investigational New Drug Application ("IND") filed by Eli Lilly and Company ("Lilly") for LY3561774, the first clinical-stage candidate to emerge from Dicerna's collaboration with Lilly. The IND milestone achievement triggers a $10 million payment to Dicerna and enables Lilly to initiate a Phase 1 clinical trial of LY3561774, which is expected before year-end 2020, for the treatment of an undisclosed cardiometabolic disease.

"The successful IND filing for LY3561774 marks an important milestone in what is expected to be the first of several resulting from our close, efficient collaboration with Lilly that could generate potentially life-saving RNAi-enabled therapies across a broad range of diseases," said Bob D. Brown, Ph.D., Chief Scientific Officer and Executive Vice President of R&D at Dicerna. "We have already initiated more than 10 discovery programs under the collaboration since closing the agreement in late 2018, providing Lilly with the potential to advance a steady cadence of RNAi product candidates to the clinic in 2021 and beyond."

The IND filing for LY3561774 is the first milestone achievement 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 disease, neurodegeneration and pain. This investigational cardiometabolic therapy and future therapies to emerge from the two companies' collaboration leverage Dicerna's proprietary RNAi technologies.

"We believe that RNAi-based medicines have the potential to be convenient and effective treatments for diseases with significant unmet need," said Andrew C. Adams, Ph.D., Vice President of New Therapeutic Modalities at Lilly. "We are pleased to have achieved what we hope will be the first of several IND filings generated from our collaboration with Dicerna and look forward to initiating the Phase 1 clinical trial of LY3561774 in the coming weeks."

This first IND filing under the collaboration agreement with Lilly triggers a $10 million milestone payment to Dicerna, which the Company received in November 2020. 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

RNA interference (RNAi) is a biologic process in which certain double-stranded RNA molecules inhibit the expression of disease-causing genes by destroying the messenger RNAs ("mRNAs") of those genes. It reflects a new approach in the development of specific and powerful therapies. Rather than targeting and binding to proteins to inhibit their activity, RNAi exerts its effects one step earlier in the gene silencing process by targeting the mRNA, the instruction set that directs the building of the protein. By attaching to this instruction set, RNAi is believed to have the ability to attack any target, including disease-causing genes that are beyond the reach of conventional antibody and small-molecule modalities. Additionally, RNAi-based therapeutic approaches hold the potential to offer more convenience for patients via infrequent dosing and a long duration of effect.

About Dicerna's RNAi Technology Platform

Dicerna's proprietary RNA interference (RNAi) technology platform, called GalXC™, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. GalXC-based compounds enable subcutaneous delivery of RNAi therapies that are designed to bind specifically to receptors on liver cells, leading to internalization and access to the RNAi machinery within the cells. The GalXC approach seeks to optimize the activity of the RNAi pathway so that it operates in the most specific and potent fashion. Dicerna has continued to innovate and is exploring new applications of its RNAi technology beyond the liver, targeting additional tissues and enabling new therapeutic applications.

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 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 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 hepatocytes, Dicerna has continued to innovate and is exploring new applications of its 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 pertaining to the Company's collaboration with Lilly, Lilly's future clinical development plans for LY3561774 and future candidates, future milestone and other payments to Dicerna, the potential of applying RNAi technology to other diseases, as well as our business and operations, including the discovery, development and commercialization of our product candidates and technology platform, and the therapeutic potential thereof, the success of our collaboration with partners and any potential future collaborations and our strategy, business plans and focus. 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. Applicable risks and uncertainties include those risks identified under the heading "Risk Factors" included in our most recent Form 10-Q filing and in other future filings with the SEC. 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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