# Lilly and Dicerna Announce RNAi Licensing and Research Collaboration

October 29, 2018

**- Companies will collaborate on RNAi research for cardio-metabolic, neurodegeneration and pain targets**
**- Dicerna to receive an upfront payment of $100 million and an equity investment of $100 million**
**- Dicerna eligible to receive up to approximately $350 million per target in development and commercialization milestones, plus royalties**

INDIANAPOLIS and CAMBRIDGE, Mass., Oct. 29, 2018 /PRNewswire/ -- Eli Lilly and Company (NYSE: LLY) and Dicerna Pharmaceuticals (NASDAQ: DRNA) today announced a global licensing and research collaboration focused on the discovery, development and commercialization of potential new medicines in the areas of cardio-metabolic disease, neurodegeneration and pain. The companies will utilize Dicerna's proprietary GalXC™ RNAi technology platform to progress new drug targets toward clinical development and commercialization. In addition, the partners will collaborate to move beyond the current technical paradigm in order to generate next-generation oligonucleotide therapeutic agents.

RNA interference (RNAi) is an emerging new approach to drug discovery, focused on a biologic process in which certain RNA molecules inhibit the expression of disease-causing genes by destroying the messenger RNAs (mRNAs) of those genes. RNAi has the potential to treat diseases by silencing some of the most well-validated, yet previously inaccessible drug targets.

"At Lilly, we go to where breaking science meets unmet medical needs," said Daniel M. Skovronsky, M.D., Ph.D., Lilly senior vice president and chief scientific officer. "We are excited to collaborate with Dicerna and utilize their RNAi expertise to study targets that up until now have proven to be very technically challenging. RNAi has the potential to treat an array of diseases that are of strategic importance to Lilly. Together with Dicerna, we aim to employ this emerging modality for greater success in drug development."

"The collaboration with Lilly provides an exceptional opportunity to leverage our proprietary GalXC platform in order to generate new medicines for cardio-metabolic diseases, and to establish a presence in new fields including neurodegeneration and pain," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Lilly, with its demonstrated leadership in each of these fields, is an ideal partner for extending the range of Dicerna's proprietary GalXC technology, which is designed to silence the expression of disease-driving genes. We are eager and ready to expand and advance our pipeline of innovative GalXC-based therapies, including both proprietary and partnered programs."

Under the terms of the agreement, Dicerna will receive an upfront payment of $100 million, as well as an equity investment of $100 million at a premium. Dicerna is also eligible to receive up to approximately $350 million per target in development and commercialization milestones, as well as tiered royalties ranging from the mid-single to low-double digits on product sales. Dicerna will work exclusively with Lilly in the neurodegeneration and pain fields, and on select targets in cardio-metabolic diseases. The two companies anticipate collaborating on more than ten targets.

This transaction is subject to clearance under the Hart-Scott-Rodino Antitrust Improvements Act and other customary closing conditions. The transaction will be reflected in Lilly's reported results and financial guidance according to Generally Accepted Accounting Principles (GAAP). There will be no change to Lilly's 2018 non-GAAP earnings per share guidance as a result of this transaction.

**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 GalXC™ RNAi Technology Platform**
The proprietary RNAi technology platform called GalXC™, invented by Dicerna, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. GalXC-based therapies are processed by the Dicer enzyme, which is the natural initiation point for RNAi within the human cell. Using GalXC, Dicerna scientists attach N-acetylgalactosamine sugars directly to the extended region of the proprietary Dicer substrate short-interfering RNA (DsiRNA) molecules, yielding multiple conjugate delivery configurations that allow flexible and efficient conjugation to the targeting ligands while stabilizing the RNAi duplex. Dicerna believes this stabilization will enable subcutaneous delivery of RNAi therapies to hepatocytes in the liver, where they are designed to specifically bind to receptors on target cells, potentially leading to internalization and access to the RNAi machinery within the cells. By using the Dicer enzyme as the entry point into RNAi, the GalXC approach seeks to optimize the activity of the RNAi pathway so that it operates in the most specific and potent fashion. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, including rare diseases, viral infectious diseases, chronic liver diseases and cardiovascular diseases.

**About Dicerna Pharmaceuticals, Inc.**
Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative, subcutaneously delivered RNAi-based therapeutics for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. Dicerna is leveraging its proprietary GalXC™ RNAi technology platform to build a broad pipeline in these core therapeutic areas, focusing on target genes where connections between target gene and diseases are well understood and documented. Dicerna intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. For more information, please visit www.dicerna.com.

**About Eli Lilly and Company**

Lilly is a global healthcare leader that unites caring with discovery to make life better for people around the world. We were founded more than a century ago by a man committed to creating high-quality medicines that meet real needs, and today we remain true to that mission in all our work. Across the globe, Lilly employees work to discover and bring life-changing medicines to those who need them, improve the understanding and management of disease, and give back to communities through philanthropy and volunteerism. To learn more about Lilly, please visit us at www.lilly.com and http://newsroom.lilly.com/social-channels. C-LLY

**Dicerna Forward-Looking Statement**
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 GalXC™; (ii) research and development plans related to GalXC; (iii) the potential of RNAi therapies for the treatment of complement-mediated diseases; and (iv) the potential for the collaboration between Lilly and Dicerna. The process by which an early stage platform such as GalXC could potentially lead to an approved product is long and subject to highly significant risks, particularly with respect to a preclinical research collaboration. Applicable risks and uncertainties include those relating to preclinical research and other risks identified under the heading "Risk Factors" included in Dicerna's most recent Form 10-Q filings 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, except as required by law.

**Lilly Forward-Looking Statement**
This press release contains forward-looking statements (as that term is defined in the Private Securities Litigation Reform Act of 1995) about the benefits of a collaboration between Lilly and Dicerna, and reflects Lilly's current beliefs. However, as with any such undertaking, there are substantial risks and uncertainties in the process of drug development and commercialization. Among other things, there can be no guarantee that Lilly will realize the expected benefits of the collaboration, or that the collaboration will yield commercially successful products. For a further discussion of these and other risks and uncertainties that could cause actual results to differ from Lilly's expectations, please see Lilly's most recent Forms 10-K and 10-Q filed with the U.S. Securities and Exchange Commission. Lilly undertakes no duty to update forward-looking statements.

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