



Boehringer Ingelheim and Dicerna Announce Collaboration to Develop Novel Treatments for Chronic Liver Diseases including Nonalcoholic Steatohepatitis (NASH)

- *Dicerna to receive up to \$201 million in upfront and success-based development and commercialization milestones, excluding royalties, for an undisclosed target*
- *The collaboration will investigate a new therapeutic approach that enables addressing previously inaccessible drug targets to protect and restore liver functionality in NASH*

INGELHEIM, Germany and CAMBRIDGE, Mass., U.S.A., November 2, 2017 – Boehringer Ingelheim and Dicerna Pharmaceuticals (NASDAQ: DRNA), a leading developer of investigational RNA interference (RNAi) therapeutics, today announced a research collaboration and license agreement to discover and develop novel GalXC™ RNAi therapeutics for the treatment of chronic liver diseases. The partnership will initially focus on nonalcoholic steatohepatitis (NASH), a devastating, chronic liver disease for which there is no approved treatment option.

NASH is caused by the buildup of fat in the liver, potentially leading to liver fibrosis and cirrhosis. It has an especially high prevalence among obese and diabetic patients, and is an area of high unmet medical need. NASH is expected to soon become the most common cause of advanced liver disorders, and it often necessitates liver transplantation.

Dicerna's GalXC technology platform uses RNAi to inhibit the expression of disease-causing genes by destroying the messenger RNAs (mRNAs) of those genes. This new approach has the potential to treat diseases by silencing previously inaccessible drug targets. It adds a further breakthrough therapy opportunity to Boehringer Ingelheim's cardiometabolic pipeline and provides additional combination options.

"We believe that Dicerna's GalXC technology platform is ideally suited for the development of novel RNAi therapies for nonalcoholic steatohepatitis and other chronic liver diseases," said Douglas M. Fambrough, President and Chief Executive Officer of Dicerna. "With strong capabilities in drug discovery, deep expertise in the cardiometabolic space, and proven commercial experience, Boehringer Ingelheim is a natural partner to speed the development of the first GalXC RNAi program targeting chronic liver disease. The collaboration combines the strong capabilities of both companies to pursue the full potential of Dicerna's GalXC technology to bring valuable and differentiated RNAi therapies to patients with liver diseases and their healthcare teams, and reflects both the promise of the GalXC technology and the strength of its underlying intellectual property."

"At Boehringer Ingelheim, our research team is diligently working to discover effective new therapies for NASH and other chronic liver diseases, which is a priority area for us," said Clive R. Wood, Ph.D., Corporate Senior Vice President Discovery Research at Boehringer Ingelheim. "This partnership complements our existing research efforts and expertise and offers distinct advantages in developing exciting new therapy options," added Wood.



Boehringer Ingelheim has a long history of excellence in the discovery and development of medicines for cardiometabolic disease patients. It has established a broad portfolio of marketed products for thromboembolic diseases, type 2 diabetes, acute myocardial infarction, hypertension, and cardio-renal risk reduction. The cardiometabolic diseases pipeline extends beyond type 2 diabetes and anticoagulation with a focus on innovative drugs for the treatment of the devastating consequences of diabetes as well as contributing factors like obesity.

Dicerna is building a portfolio of research and development programs to advance the treatment of diseases involving the liver, including chronic liver diseases, viral infectious diseases, cardiovascular diseases and rare diseases. The company aims to leverage its proprietary GalXC technology to develop innovative RNAi therapeutics to positively transform the lives of individuals living with these diseases.

Under the terms of the agreement, Dicerna may receive more than \$200 million from Boehringer Ingelheim, including an upfront payment, development and commercial milestone payments, and research and development reimbursement for a GalXC candidate product addressing an undisclosed NASH target. Dicerna is also eligible to receive royalties staggered up to double-digits on worldwide net sales.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative RNAi-based therapeutics for diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company 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. The Company 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 the GalXC™ RNAi Technology Platform

GalXC™ is a proprietary technology platform invented by Dicerna to discover and develop next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, including rare diseases, chronic liver diseases, cardiovascular disease and viral infectious diseases. Using GalXC, Dicerna's scientists attach N-acetylgalactosamine sugars directly to the extended region of our proprietary Dicer substrate short-interfering RNA (DsiRNA) molecules, yielding multiple proprietary conjugate delivery configurations. Many of the conjugates produced via GalXC incorporate a folded motif known as a tetraloop in the extended region. Our tetraloop configuration, which is unique to Dicerna's GalXC compounds, allows flexible and efficient conjugation to the targeting ligands, and stabilizes the RNAi duplex which the Company believes will enable subcutaneous delivery of its 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. The technology may offer several distinct benefits, as suggested by strong preclinical data. These benefits include: potency that is on par with or better than comparable platforms; highly specific binding to gene targets; long duration of action; and an infrequent subcutaneous dosing regimen.

Dicerna owns and has licensed a portfolio of patents, patent applications and other intellectual property covering: (1) certain aspects of the structure and uses of DsiRNA and GalXC molecules, including their manufacture and use as therapeutics, and RNAi-related mechanisms, (2) chemical modifications to DsiRNA and GalXC molecules that improve their properties and suitability for therapeutic uses, (3) DsiRNA and GalXC molecules directed to specific gene sequences and drug targets as treatments for particular diseases and (4) delivery technologies, such as in the field of lipid nanoparticles and lipid nanoparticle formulation, and chemical modifications such as conjugation to targeting moieties.

About Boehringer Ingelheim

Innovative medicines for people and animals have for more than 130 years been what the research-driven pharmaceutical company Boehringer Ingelheim stands for. Boehringer Ingelheim is one of the pharmaceutical industry's top 20 companies and to this day remains family-owned. Day by day, some 50,000 employees create value through innovation for the three business areas, human pharmaceuticals, animal health and biopharmaceutical contract manufacturing. In 2016, Boehringer Ingelheim achieved net sales of around 15.9 billion euros. With more than three billion euros, R&D expenditure corresponds to 19.6 per cent of net sales.

Social responsibility comes naturally to Boehringer Ingelheim. That is why the company is involved in social projects such as the "Making More Health" initiative. Boehringer Ingelheim also actively promotes workforce diversity and benefits from its employees' different experiences and skills. Furthermore, the focus is on environmental protection and sustainability in everything the company does. More information about Boehringer Ingelheim can be found on www.boehringeringelheim.com or in our annual report: <http://annualreport.boehringeringelheim.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 GalXC™; (ii) research and development plans related to GalXC; and (iii) the potential of our technology and drug candidates in our research and development pipeline. 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 pre-clinical research collaboration. Applicable risks and uncertainties include those relating to our preclinical research and other 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.



Contacts for Boehringer Ingelheim:

Dr. Reinhard Malin
Head of Communications Innovation Unit
Boehringer Ingelheim Corporate Center GmbH Media + PR
reinhard.malin@boehringeringelheim.com
P: +49 6132 77-90815 :: M: + 49 151 150 20 690
www.boehringeringelheim.com

Linda Ruckel
Associate Director, Media and Corporate Reputation
Boehringer Ingelheim Pharmaceuticals, Inc.
Ridgefield, Connecticut
linda.ruckel@boehringeringelheim.com
P: 203-791-6672 :: C: 203-957-5114
www.boehringeringelheim.com

Contacts for Dicerna Pharmaceuticals:

Paula Schwartz
Managing Director
Rx Communications Group
pschwartz@rxir.com
P: 917-322-2216
www.rxir.com

Alex Van Rees
Account Director
SmithSolve Communications
alex.vanrees@smithsolve.com
P: 973-442-1555 ext. 111
www.smithsolve.com