



Boehringer Ingelheim Exercises Option on Second Hepatic Disease Target from Research Collaboration with Dicerna

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Collaboration seeks to develop novel treatments for chronic liver diseases, including nonalcoholic steatohepatitis (NASH)

INGELHEIM, Germany & CAMBRIDGE, Mass.--([BUSINESS WIRE](#))--[Boehringer Ingelheim](#) and [Dicerna Pharmaceuticals](#)(NASDAQ:DRNA), a leading developer of investigational RNA interference (RNAi) therapeutics, today announced that [Boehringer Ingelheim](#) has exercised an option to receive exclusive rights to a second hepatic disease target emerging from its research collaboration and license agreement with Dicerna. The collaboration, established in October 2017, aims to discover and develop novel GalXC™ RNAi therapeutics for the treatment of chronic liver diseases, with an initial focus on nonalcoholic steatohepatitis (NASH), a devastating disease for which there is no approved treatment.

The option is the second target under the two companies' research collaboration and license agreement. Under the terms of the agreement, [Boehringer Ingelheim](#) will be responsible for future clinical development and commercialization of the therapeutic target. Dicerna is eligible to receive development and commercial milestone payments, and royalties on worldwide net sales.

"It is extremely gratifying for [Boehringer Ingelheim](#) to exercise its option for a second therapeutic candidate from our productive research collaboration," said Douglas M. Fambrough, President and Chief Executive Officer of Dicerna. "The option exercise underscores the potential of Dicerna's GalXC technology platform in facilitating the discovery and development of novel RNAi therapies for nonalcoholic steatohepatitis and other chronic liver diseases. We look forward to continued progress and the potential advancement of clinical candidates from our partnership."

Dicerna and [Boehringer Ingelheim](#) selected the target based on its ability to be drugged using Dicerna's proprietary GalXC technology platform. The GalXC platform uses RNAi to inhibit the expression of disease-causing genes by destroying the messenger RNAs (mRNAs) of those genes. The approach has the potential to treat diseases by silencing previously inaccessible drug targets.

"[Boehringer Ingelheim](#) is pleased with the progress of our initial collaboration with Dicerna and based on early findings and our strong relationship, we are expanding our research efforts," said Clive R. Wood, Ph.D., Corporate Senior Vice President Discovery Research at [Boehringer Ingelheim](#). "Today's announcement exemplifies our commitment to discovering new therapies for NASH and other chronic liver diseases and advances our research efforts in this priority area."

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 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.

About Dicerna's GalXC™ RNAi Technology Platform

Dicerna's proprietary 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 therapies are processed by the Dicer enzyme, which is the natural initiation point for RNAi within the human cell. Using GalXC, Dicerna attaches 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, chronic liver diseases, cardiovascular diseases, and viral infectious 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, chronic liver diseases, cardiovascular diseases, and viral infectious 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. Dicerna has strategic collaborations with [Boehringer Ingelheim](#), [Eli Lilly and Company](#), and [Alexion Pharmaceuticals](#). For more information, please visit www.dicerna.com.

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.boehringer-ingelheim.com or in our annual report: <http://annualreport.boehringer-ingelheim.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 Dicerna makes 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 Dicerna's preclinical research and other risks identified under the heading "Risk Factors" included in Dicerna's 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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