Dicerna Announces Boehringer Ingelheim's Acceptance of Candidate for Development Under RNAi Research Collaboration and License Agreement

May 24, 2021

Development Candidate Will Be Evaluated for Treatment of Nonalcoholic Steatohepatitis

Candidate Selection Triggers Single-Digit Multimillion USD Milestone Payment to Dicerna

LEXINGTON, Mass.--(BUSINESS WIRE)--May 24, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced that Boehringer Ingelheim has accepted a GalXC™ RNAi candidate for advancement under the existing agreement between the companies for the discovery and development of novel therapies for the treatment of chronic liver diseases. Referred to as DCR-LIV2, the compound will be investigated for the treatment of nonalcoholic steatohepatitis (NASH), a chronic liver disease for which there are no approved therapeutic interventions. Acceptance of DCR-LIV2 as a development candidate triggered a single-digit multimillion USD preclinical milestone payment to Dicerna, which the Company expects to receive in the second quarter of 2021.

"Advancing this selected target to development demonstrates the commitment of our team and our collaborative partners at Boehringer Ingelheim to discovering new and innovative ways to treat NASH using RNAi," said Bob D. Brown, Ph.D., Dicerna's Chief Scientific Officer and Executive Vice President of R&D. "Dicerna's GalXC technology is the ideal platform for this vital work. What's more, this announcement marks an important milestone for Dicerna and highlights the productivity of our RNAi discovery research engine, as all of Dicerna's discovery-oriented collaborations have now produced GalXC-based development candidates that are advancing toward the clinic or have already entered clinical development."

Under the terms of the existing agreement between Dicerna and Boehringer Ingelheim, Dicerna is eligible to receive up to $170.0 million in potential additional development and commercial milestones related to DCR-LIV2. Dicerna is also eligible to receive tiered mid-single-digit royalties on potential global net sales.

About Nonalcoholic Steatohepatitis (NASH)

Nonalcoholic steatohepatitis (NASH) is a condition characterized by the buildup of fat in the liver, potentially leading to liver fibrosis, cirrhosis, liver failure or cancer, and has an especially high prevalence among people who are obese or have type 2 diabetes. NASH is estimated to affect approximately 1.5% to 6.5% of adults in the U.S.[1]

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 targeted destruction of the messenger RNA (mRNA) made from the gene. Rather than seeking to inhibit a protein directly, 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 for 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 Boehringer Ingelheim International GmbH and the therapeutic potential of the development candidate under such collaboration. 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.

[1] Definition & Facts of NAFLD & NASH | NIDDK (nih.gov) accessed May 21, 2021.

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