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Dicerna Prioritizes Resources to Advance GalXC™ Product Candidates

Primary Hyperoxaluria Development Program Transitioned From DCR-PH1 to Subcutaneously Delivered GalXC Candidate DCR-PHXC

Clinical Development Discontinued for DCR-MYC in Oncology Indications

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Dicerna Pharmaceuticals, Inc. (Nasdaq:DRNA), a leading developer of investigational RNA interference (RNAi) therapeutics, today announced that the Company will focus its resources on its proprietary GalXC™ technology platform to advance development of product candidates in its core therapeutic areas of rare diseases, chronic liver diseases, cardiovascular disease and viral infectious diseases. Under this plan, Dicerna will transition its primary hyperoxaluria (PH) development program to focus on DCR-PHXC, a subcutaneously delivered GalXC clinical candidate, which was announced earlier this year. The Company also announced that it will discontinue clinical development of DCR-MYC, a DsiRNA-based therapeutic formulated as an EnCore™ lipid nanoparticle (LNP) for delivery to solid tumors, because preliminary results do not meet the Company's expectations for further development.

"Based on the performance of the GalXC platform, the strength of the preclinical data and the broad therapeutic opportunities for RNAi in liver-targeted diseases, we are prioritizing resources to advance the product candidates emerging from this platform," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Discontinuing our DCR-PH1 and DCR-MYC lipid nanoparticle programs allows us to focus our resources on efficiently developing our GalXC product candidates and building on the solid scientific foundation in RNAi that Dicerna has developed over the past decade. We greatly appreciate the participation of all of the patients, families and clinical investigators in the DCR-PH1 development program, as their contributions provided important insights into the primary hyperoxaluria disease state, which will guide the development of DCR-PHXC."

The GalXC platform is a fully enabled RNAi drug discovery engine with potentially powerful capabilities that the Company believes could result in potency that is on par with or better than comparable platforms. As Dicerna reported during its recent Investor Day, subcutaneously delivered GalXC compounds silenced 12 different disease targets in animal models, highlighting the long duration of action, infrequent dosing and tolerability of GalXC-based compounds. Use of the GalXC platform yielded gene silencing of greater than 90% for multiple genes in non-human primates (NHPs) after a single dose. In an NHP model of an undisclosed rare disease gene target, a single 3 mg/kg dose achieved a maximum gene silencing of 94%, with an average gene silencing of approximately 88%. Another single 3 mg/kg dose NHP study resulted in an average of 97% silencing of an undisclosed rare disease gene target. Based on this evidence, Dicerna believes that DCR-PHXC has the potential to be a new treatment option for patients with PH.

In addition to DCR-PHXC, which is in preclinical development, Dicerna expects to launch two more GalXC programs in 2016: one will focus on cardiovascular disease targeting PCSK9; the other is an undisclosed rare disease program. Dicerna expects to launch three additional programs annually, with the intent to advance five programs into the clinic by 2019.

GalXC compounds offer several unique characteristics, including:

- Longer RNAi duplexes (i.e., compared to standard RNAi molecules) provide greater potential to increase potency and reduce toxicity, using a toolbox of standard oligonucleotide chemistries.
- A unique tetraloop configuration stabilizes the RNA duplex, provides multiple points for addition of GalNAc sugars and interfaces effectively with the RNAi machinery within target cells.

The GalXC platform enables rapid discovery and efficient advancement of research activities. Within 30 days of nominating a gene target, Dicerna can design, synthesize and validate an *in vivo* GalXC construct.

PH1 Program Transition

Dicerna will transition its PH development program to focus on DCR-PHXC and expects to file an IND or CTA for DCR-PHXC in late 2017. The Company will discontinue the development program for DCR-PH1, an investigational therapy formulated in an LNP delivery system obtained through a licensing agreement with Arbutus Biopharma Corporation (formerly known as Tekmira Pharmaceuticals Corporation). DCR-PH1 was being studied in two clinical trials, DCR-PH1-101 in patients with

primary hyperoxaluria type 1 (PH1) and DCR-PH1-102 in normal healthy volunteers (NHVs).

Data from the DCR-PH1-102 clinical trial, in which 21 NHVs were randomized to receive DCR-PH1 at a dose of 0.005, 0.015 and 0.05 mg/kg or placebo, showed an increase in urine glycolate levels, a biomarker of DCR-PH1 treatment activity, in the top two DCR-PH1 dosing groups. Those data, which were presented on Sept. 22, 2016, at the 17th Congress of the International Pediatric Nephrology Association in Iguaçu, Brazil, provide the proof of concept for the pharmacological activity of RNAi-based therapy in PH. Based on the DCR-PH1 proof-of-concept data in humans, the utility of the GalXC platform, and the DCR-PHXC preclinical data, the Company believes DCR-PHXC has the potential to be a better therapeutic candidate for patients with PH.

"The encouraging data from Dicerna give us hope that research on a GalXC-based therapeutic agent can potentially benefit patients living with primary hyperoxaluria, a devastating disease that often causes early-onset renal failure," said Craig Langman, M.D., a pediatric nephrologist and the Isaac A. Abt, M.D. Professor of Kidney Diseases at the Feinberg School of Medicine, Northwestern University and Head, Kidney Diseases, at Lurie Children's Hospital. "There is a significant unmet medical need for a viable therapy for patients with primary hyperoxaluria, as the current treatment option consists of combined transplantation of the kidney and liver, a highly invasive procedure with significant morbidity."

As a part of the Company's ongoing commitment to the PH1 community, Dicerna will continue to advance its **P**rimary **HY**peroxaluria **O**bservational **S**tudy (PHYOS), which is collecting data on key biochemical parameters implicated in the pathogenesis of PH1. The Company hopes to use these data to better understand the baseline PH1 disease state, knowledge that will help guide long-term drug development plans.

"We look forward to continuing to work with Dicerna as the Company investigates a potential therapeutic option for the PH community, and we appreciate the Company's decision to move forward with a new investigational therapy based on recent scientific advances," said Kim Hollander, executive director of the Oxalosis and Hyperoxaluria Foundation. "We encourage patients and families affected by PH to learn more about clinical trials and international patient registries, which we hope will strengthen our understanding of PH for potential therapies in the future."

DCR-MYC Program Discontinuation

Dicerna will discontinue the clinical development program for DCR-MYC, which was being investigated in two clinical trials: DCR-MYC-101, a Phase 1 trial in patients with advanced solid tumors and hematological malignancies, including an expansion cohort in patients with pancreatic neuroendocrine tumors; and DCR-MYC-102, a Phase 1b/2 trial in patients with advanced hepatocellular carcinoma (HCC). MYC is an oncogene frequently amplified or overexpressed in a wide variety of tumor types.

While preliminary data from the DCR-MYC-101 trial provided evidence of clinical response and molecular knockdown of MYC in patients, the early efficacy results do not meet the Company's expectations to warrant further development. Paired tumor biopsies pre- and post-treatment showed proof of concept with drug delivery and provided clear evidence of RNAi-mediated MYC messenger RNA destruction in tumors from all patients tested; however, the level of MYC knockdown was below the level of molecular knockdown that the Company targeted. In the HCC trial, topline findings showed that a dose of up to 0.85 mg/kg was well tolerated; however, no clinical activity (based on the Modified Response Evaluation Criteria in Solid Tumors criteria) has been observed to date.

Dicerna will present DCR-MYC clinical and molecular data at the 12th Annual Meeting of the Oligonucleotide Therapeutics Society in Montreal, Quebec on September 28, 2016.

DCR-BCAT Program Update

In addition to DCR-MYC, Dicerna has a second oncology program, DCR-BCAT, which targets the WNT-beta-catenin pathway. Given the Company's focus on advancing its GalXC-based programs, Dicerna will seek strategic alternatives to further develop DCR-BCAT, which employs an improved and enhanced EnCore LNP delivery capability, compared to earlier versions of the technology.

About GalXC™

GalXC™ is a proprietary technology platform invented by Dicerna to advance the evaluation of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. Compounds produced via the GalXC technology 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 scientists attach N-acetylgalactosamine (GalNAc) sugars directly to the extended region of a Dicer substrate short-interfering RNA (DsiRNA-EX) molecule, yielding multiple proprietary conjugate delivery configurations. GalXC enables subcutaneous delivery of Dicerna's 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 dosing regimen. Conjugates produced via the GalXC platform can be administered as simple saline solutions and do not need transport technologies (such as lipid nanoparticles) to facilitate delivery.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc., is an RNA interference-based biopharmaceutical company focused on the discovery and development of innovative treatments for rare, inherited diseases involving the liver and for other therapeutic areas in which the liver plays a key role. The Company is using its proprietary RNA interference (RNAi) technology platform to build a broad pipeline in these therapeutic areas. In many cases, Dicerna is pursuing targets that have historically been difficult to inhibit using conventional approaches, but where connections between targets and diseases are well understood and documented. The Company intends to discover, develop and commercialize these novel therapeutics either on its own or in collaboration with pharmaceutical partners. For more information, please visit www.dicerna.com.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements, including, for example, potential therapeutic and drug discovery capabilities, our expected timeline of development and licensing plans. 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 relating to our clinical and 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.

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