November 7, 2016

Dicerna Reports Third Quarter 2016 Financial and Operational Results

Management to Host Conference Call Today at 4:30 p.m. ET

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA), a leading developer of investigational RNA interference (RNAi) therapeutics, today reported financial and operational results for the third quarter ended September 30, 2016.

"This quarter, we made the strategic decision to focus our resources on advancing our GalXC™ subcutaneous RNAi technology platform and to prioritize the product candidates emerging from this powerful product engine," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "Our decision was based on the strength of the GalXC preclinical data and broad opportunities this technology enables in our core therapeutic areas of rare disease, chronic liver diseases, cardiovascular diseases and liver infectious diseases. In preclinical models across these areas, the potency and duration of action results for the GalXC platform have consistently supported the potential for target gene silencing in the liver with a simple, infrequent, subcutaneous injection paradigm. As a result, we transitioned our primary hyperoxaluria program to the GalXC-based DCR-PHXC from DCR-PH1, as we believe DCR-PHXC has the potential to be a better therapeutic candidate for patients with this disease. We also discontinued our DCR-MYC program in oncology because early efficacy results did not meet our threshold for further development. We believe this strategic shift will put us in a stronger position to execute our development strategy more efficiently and build an exciting pipeline of subcutaneously-delivered, RNAi-based therapeutics with clear differentiation and that have the potential to be first-in-class in specific indications."

GalXC™ Program Update

- Primary Hyperoxaluria: Dicerna is developing DCR-PHXC for the treatment of primary hyperoxaluria (PH), a rare inborn error of metabolism in which the liver produces excessive levels of oxalate, which in turn causes damage to the kidneys and to other tissues in the body. DCR-PHXC is in preclinical development and is advancing into Investigational New Drug (IND)-enabling studies. Dicerna intends to file an IND submission or Clinical Trial Application (CTA) for DCR-PHXC in late 2017 and commence human clinical trials shortly thereafter.

  To facilitate DCR-PHXC development, Dicerna continues to advance its **P**rimary **HY**peroxaluria **O**bservational **S**tudy (PHYOS), an international, multicenter, observational study in patients with a genetically confirmed diagnosis of PH1. PHYOS is collecting data on key biochemical parameters implicated in the pathogenesis of PH1. Dicerna hopes to use the data to better understand the baseline PH1 disease state, which will help guide long-term drug development plans.

- Cardiovascular Disease: Dicerna is using its GalXC RNAi platform to develop an investigational therapeutic that targets PCSK9 to treat statin-refractory patients with hypercholesterolemia. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform has the potential to produce a PCSK9-targeted therapy with more attractive commercial properties than existing monoclonal antibody therapies, based on smaller subcutaneous injection volumes and less frequent dosing, while providing equal or superior control of serum cholesterol. The Company is continuing to advance this program and is on track to nominate a candidate for preclinical development in 2016.
- Undisclosed Rare Disease: Dicerna is developing a GalXC-based therapeutic that targets a liver-expressed gene involved in a rare disease associated with high morbidity and mortality. This undisclosed program is on track for formal program launch with an optimized lead candidate in 2016.

Dicerna has the capacity to launch up to three additional programs annually, with the intent to advance five programs into the clinic by the end of 2019. The Company expects to initiate programs for hepatitis B virus (HBV) as well as two additional programs from its core therapeutic areas in 2017.

Legacy Program Update

DCR-PH1

- During the third quarter of 2016, Dicerna transitioned its PH program to DCR-PHXC from DCR-PH1. The Company discontinued the development program for DCR-PH1, an investigational therapy formulated in a lipid nanoparticle

(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 Phase 1 clinical trials: DCR-PH1-101 in patients with primary hyperoxaluria type 1 (PH1) and DCR-PH1-102 in normal healthy volunteers (NHV).

- On September 22, 2016, Dicerna presented initial Phase 1 data from the DCR-PH1-102 study at the 17th Congress of the International Pediatric Nephrology Association (IPNA) in Iguaçu, Brazil. Data from DCR-PH1-102, 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. Dicerna believes these data provide the proof of concept for the pharmacological activity of RNAi-based therapy in PH.

Oncology Program Update

DCR-MYC

- During the third quarter of 2016, Dicerna announced it discontinued the clinical development program for DCR-MYC, which was being investigated as a specific inhibitor of MYC, an oncogene frequently amplified or overexpressed in a wide variety of tumor types. DCR-MYC, a DsiRNA-based therapeutic formulated in Dicerna's EnCore™ lipid nanoparticle for delivery to solid tumors, was being studied 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). Dicerna presented DCR-MYC clinical trial results at the 12th Annual Meeting of the Oligonucleotide Therapeutics Society on September 28, 2016.
  - While clinical response and molecular knockdown of MYC patients was observed in preliminary data from the DCR-MYC-101 trial, the early efficacy results did 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 DCR-MYC-102 trial in patients with HCC, a dose of up to 0.85 mg/kg met the Company's expectations for safety; however, no clinical activity (based on the modified Response Evaluation Criteria in Solid Tumors criteria) was observed.

Financial Results

- **Cash Position** - As of September 30, 2016, Dicerna had $57.5 million in cash and cash equivalents and held-to-maturity investments as compared to $94.6 million in cash and cash equivalents and held-to-maturity investments as of December 31, 2015. In addition, the Company had $1.1 million of restricted cash, which reflects collateral securing its lease obligations.
- **R&D Expenses** - Research and development (R&D) expenses for the third quarter were $10.1 million, compared to $12.1 million for the same period in 2015. The decrease was due to a reduction in manufacturing and toxicology testing activities and a decrease in discovery and early development costs, as programs advanced year over year into clinical testing, partially offset by an overall increase in pre-clinical studies for Dicerna's GalXC platform and an increase in clinical trial activities. Employee-related expenses and facilities, depreciation and other expenses have remained consistent.
- **G&A Expenses** - General and administrative (G&A) expenses for the third quarter were $4.3 million, compared to $4.9 million for the same period in 2015. The decrease was due to a decrease in stock-based compensation and a decrease in other general and administrative expenses. Dicerna expects G&A expenses to increase in the future as the Company continues to expand its operating activities and incur additional costs associated with being a publicly-traded company.
- **Net Loss** - Net loss for the third quarter was $14.2 million compared to a net loss of $16.9 million for the same period in 2015.

For more detailed information and analysis see Dicerna's Quarterly Report on Form 10-Q, which was filed with the Securities and Exchange Commission (SEC) on November 7, 2016.

Guidance

Based on Dicerna's current cash position and operating plan, the Company reiterates its expectation that it has sufficient cash to fund planned operations for at least the next 12 months. This estimate assumes no additional funding from new partnership agreements or debt or equity financing events.

Conference Call

Management will conduct a conference call at 4:30 p.m. ET today to review the Company's third quarter 2016 financial results and provide a general business update. The conference call can be accessed by dialing (855) 453-3834 or (484) 756-4306 (international), and referencing conference ID 10030267 prior to the start of the call. The call will also be webcast via the Internet and will be available under the "Investors & Media" section of the Dicerna website, www.dicerna.com. A replay of the call will be available beginning at 7:30 p.m. ET on November 7, 2016. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 10030267. The webcast will also be archived on the Company's website.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative RNA interference (RNAi)-based therapeutics for rare diseases involving the liver and for other diseases involving the liver such as chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company is using its proprietary GalXC™ RNAi technology platform to build a broad pipeline in these core therapeutic areas. Dicerna is pursuing target genes where connections between target gene 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.

About 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 scientists attach N-acetylgalactosamine (GalNAc) sugars directly to the extended region of our proprietary Dicer substrate short-interfering RNA 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. The 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 we believe will enable 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 subcutaneous dosing regimen.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements, including, for example, our expected timeline and plans for development, potential collaborations, and potential therapeutic benefits. 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.

Dicerna Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets (Unaudited)
(In thousands)

	September 30, 2016	December 31, 2015
Cash and cash equivalents	$ 32,544	$ 56,058
Held-to-maturity investments	$ 25,002	$ 38,551
Total assets	$ 62,949	$ 100,023
Total liabilities	$ 9,817	$ 9,001
Total stockholders' equity	$ 53,132	$ 91,022

Condensed Consolidated Statements of Operations (Unaudited)
(In thousands, except share and per share data)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
Revenues	$ 162	-	$ 162	184
Operating expenses:				
Research and development	10,061	$ 12,142	32,357	$ 32,708
General and administrative	4,338	4,857	13,478	14,822
Total operating expenses	14,399	16,999	45,835	47,530
Loss from operations	(14,237)	(16,999)	(45,673)	(47,346)
Interest income	61	55	182	142
Net loss	$ (14,176)	$ (16,944)	$ (45,491)	$ (47,204)
Net loss per share - basic and diluted	$ (0.68)	$ (0.82)	$ (2.20)	$ (2.47)
Weighted average shares outstanding - basic and diluted	20,752,416	20,592,840	20,708,600	19,097,230

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