August 4, 2016

Dicerna Reports Second 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 second quarter ended June 30, 2016.

"This quarter marks a significant strategic step forward for Dicerna as we formally introduced our GalXC™ subcutaneous delivery platform at our June Investor Day. During that event we showed consistent, potent and durable preclinical gene silencing data from GalXC molecules against 12 different disease-associated genes, including six examples of data from non-human primates, to highlight the robust basis for further investigation of the silencing of disease-causing genes in the liver across multiple therapeutic areas," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Our GalXC platform is now a fully-enabled RNAi drug discovery engine with powerful capabilities that could result in potency that is on par with or better than comparable platforms, longer duration of action versus other modalities, exquisite specificity to gene targets, and a simple, infrequent dosing regimen. We are launching three GalXC research programs in 2016, the first in primary hyperoxaluria, a second in cardiovascular disease targeting PCSK9, and a third in an undisclosed orphan genetic disease. We are also continuing to advance our DCR-PH1 and DCR-MYC clinical research programs."

Technology Update

Subcutaneous delivery to the liver with GalXC Platform: Dicerna's proprietary GalXC platform utilizes the Company's extended dicer substrate short interfering RNA (DsiRNA-EX) technology conjugated to a targeting agent to enable delivery to the liver via subcutaneous administration. Dicerna scientists attach small drug delivery agents, known as N-acetylgalactosamine (GalNAc) sugars, directly to the extended region of a DsiRNA-EX molecule, a chemically optimized, double stranded RNA developed by Dicerna. The GalNAc sugars are designed to specifically bind to receptors on hepatocyte target cells in the liver, potentially leading to effective delivery and silencing of specific gene targets within the cells. Many of the conjugates produced using the GalXC platform incorporate a folded motif known as a tetraloop, which stabilizes the RNA duplex and provides multiple conjugation points for the addition of the GalNAc sugars. The tetraloop configuration, which is proprietary and unique to Dicerna's conjugates, interfaces effectively with the RNAi machinery within target cells.

- On June 29, 2016, Dicerna held its Investor Day in New York City where it presented robust preclinical data for multiple gene targets, which provide a basis for further investigating the broad application of its fully enabled GalXC RNAi therapeutic engine, outlined its development strategy and expansive therapeutic opportunities, and announced its plan to launch three GalXC research programs in 2016.
  - Data highlights from Investor Day included:
    - GalXC compounds delivered subcutaneously silenced 12 different disease targets, including six in non-human primates (NHPs), providing a basis for further investigation of the GalXC system in a variety of disease targets.
    - Use of the GalXC platform yielded gene silencing of greater than 90% for multiple genes in NHPs after a single dose. In a NHP model, a single 3 mg/kg dose achieved the maximum HAO1 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.
    - Data regarding safety, tolerability profile, and duration of effect provide a basis for further investigating the feasibility of a monthly preclinical dosing interval, or even less frequently for select gene targets.
    - Preclinical data regarding GalXC-mediated efficacy was reported across several therapeutic areas, including multiple rare diseases and three different rodent models for chronic liver diseases.
  - Dicerna described the large number of opportunities for RNAi therapies in its core areas of therapeutic focus including rare diseases, chronic liver diseases such as NASH and fatty liver disease, cardiovascular diseases, and viral infectious diseases such as hepatitis B virus. In all, Dicerna has qualified 29 disease targets for further investigation in these disease areas, including both first-in-class and validated targets. The Company expects its work on these targets will create extensive opportunities for partnership and collaboration as well populating its internal rare disease pipeline over the coming quarters and years.

- Dicerna expects to launch three GalXC research programs in 2016 and multiple programs in 2017 and beyond, with the intent to have five programs in the clinic by 2019. DCR-PHsc, Dicerna's first GalXC program in primary hyperoxaluria, is advancing toward IND-enabling studies. The Company expects to file an IND/CTA for DCR-PHsc in late 2017. Dicerna will also launch a program in cardiovascular disease targeting PCSK9 as well as a rare disease program later this year.

Rare Disease Program Update

DCR-PH1: DCR-PH1 is an intravenously infused DsiRNA-EX-based therapeutic candidate for primary hyperoxaluria type 1 (PH1), a severe, rare genetic disease of liver metabolism that often results in life-threatening damage to the kidneys. In a genetic mouse model of PH1, DCR-PH1 knocked down the activity of the HAO1 gene transcript that encodes for the enzyme glycolate oxidase, thereby reducing the production of oxalate, the key mediator of disease pathology in PH1. Similar results, if obtained in PH1 patients, may have significant clinical benefit. In non-human primate studies, a single dose of DCR-PH1 led to an average of 94% knockdown, with a maximum of 96% knockdown, of the HAO1 gene transcript. The DCR-PH1 clinical research program consists of the following studies:

- PH1 Patient Study: During the second quarter of 2016, Dicerna dosed the first patient in its DCR-PH1-101 trial, the first Phase 1 clinical trial of DCR-PH1 in patients with PH1, in Germany. The DCR-PH1-101 clinical trial is testing single ascending doses of DCR-PH1 in patients who have a genetically confirmed diagnosis of PH1. Investigators will monitor patients for changes in urinary and plasma glycolate and oxalate, key efficacy markers in PH1.
- PHYOS: Dicerna continues to enroll PH1 patients in PHYOS (Primary HYperoxaluria Observational Study), an international, multicenter, observational study in patients with a genetically confirmed diagnosis of PH1. To date, 18 patients have been enrolled in the study. PHYOS is designed to measure biomarkers implicated in PH1 and to identify patients who may be eligible for the Phase 1 trial of DCR-PH1. The trial was initiated in the fourth quarter of 2015.
- Healthy Volunteer Study: Dicerna continues to enroll participants in DCR-PH1-102, a Phase 1 dose escalation trial of DCR-PH1 in healthy volunteers. The primary objective of this study is to determine the safety profile of DCR-PH1 in healthy volunteers in order to support dosing of PH1 patients in the United States. The trial was initiated in the fourth quarter of 2015.

Oncology Program Update

DCR-MYC: DCR-MYC is a being investigated as a specific inhibitor of MYC, an oncogene frequently amplified or overexpressed in a wide variety of tumor types, including hepatocellular carcinoma (HCC). MYC has long been considered "undruggable" with small molecule and antibody technologies. DCR-MYC is a DsiRNA-based therapeutic formulated in Dicerna's EnCore™ lipid nanoparticle for delivery to solid tumors. In preclinical studies, DCR-MYC knocked down MYC transcript levels and reduced tumor volume in multiple mouse tumor models, including models of HCC. DCR-MYC is currently being tested in two clinical trials.

- Dicerna completed the dose-escalation phase of the Phase 1 DCR-MYC all-comers trial and will enroll patients in its two expansion cohorts of this trial through the end of August. The first expansion cohort, in patients with low-to-intermediate grade pancreatic neuroendocrine tumors (PNETs) having failed one or two lines of prior therapy, was selected as a focus area after observing two clinical responses in PNET patients during dose escalation, out of three PNET patients treated. The second expansion cohort is in patients undergoing pre- and post-treatment biopsies in order to directly assess molecular markers of RNAi activity against the MYC transcript. Direct observation of RNAi activity of DCR-MYC, combined with observations both of anti-tumor activity and inhibition of FDG uptake in tumors, will potentially establish proof-of-concept for the RNAi-based mechanism of action of DCR-MYC. The Company expects to have data for both of these expansion cohorts by the end of 2016.
- Dicerna's Phase 1b/2 clinical trial of DCR-MYC in patients with advanced HCC has achieved a dosing level of 0.85 mg/kg. While an objective response based on modified Response Evaluation Criteria in Solid Tumors (mRECIST) has not been observed, a reduction in circulating alpha-fetoprotein level, a marker associated with anti-tumor activity, has been observed. Dicerna expects to have preliminary data from this trial by the end of 2016. The trial was initiated in December 2014.
- There have been no serious adverse events (SAEs) that investigators have attributed to treatment with DCR-MYC; the adverse event (AE) profile indicates most events have been mild or moderate in severity, not related to treatment with DCR-MYC, and mostly due to the progression of disease.

Corporate Update

- On June 15, 2016, Dicerna announced the appointment of Martin Freed, M.D., former co-founder and chief medical officer of Civitas Therapeutics, to its board of directors. The appointment was effective immediately and increased the size of Dicerna's board of directors to eight members. Dr. Freed is an independent director.

Dr. Freed has served as an independent consultant to several private pharmaceutical, biotechnology, and healthcare companies, specializing in clinical and general pharmaceutical development and clinical and regulatory strategy since February 2015. He was co-founder and chief medical officer of Civitas Therapeutics, Inc., from December 2010 to October 2014 (acquired by Acorda Therapeutics, Inc., or Acorda) and senior vice president, clinical development of Acorda from October 2014 through January 2015. In addition, Dr. Freed has served as chief medical officer and has provided strategic and operational planning and execution, as well as medical leadership for clinical pharmacology and development strategy and preclinical development for multiple pharmaceutical companies throughout his career. These companies include Avila Therapeutics, Inc., Taligen Therapeutics, Adnexus Therapeutics, Inc., (acquired by Bristol-Myers Squibb), and Vitae Pharmaceuticals, Inc. Prior to Vitae, he spent 14 years at GlaxoSmithKline and its predecessor, SmithKline Beecham Pharmaceuticals, or SmithKline Beecham.

Dr. Freed has been Board Certified in Internal Medicine, Nephrology and Clinical Pharmacology. He performed his internal medicine residency at Temple University Hospital and nephrology fellowship at Yale-New Haven Hospital. A Fellow of the American College of Physicians, Dr. Freed received his B.S. with Distinction in Biology from the University of Delaware and M.D. from Pennsylvania State University's College of Medicine.

Financial Results

- **Cash Position** - As of June 30, 2016, the Company had $69.2 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 second quarter were $11.0 million, compared to $11.9 million for the same period in 2015. The decrease in R&D expenses was due primarily to the timing of manufacturing and pre-clinical activities related to our PH-1 IND filing as well as a decrease in platform-related expenses due to a lower spending in discovery and early development as programs have advanced into clinical and manufacturing. These decreases were partially offset by an overall increase in clinical activities from initiating additional sites and enrolling patients in clinical trials and increased employee-related expenses primarily due to additional hiring.
- **G&A Expenses** - General and administrative (G&A) expenses for the second quarter were $4.7 million, compared to $4.5 million for the same period in 2015. The increase was primarily due to higher employee-related expenses, including additional hiring to support operations.
- **Net Loss** - Net loss for the second quarter was $15.6 million compared to a net loss of $16.2 million for the same period in 2015.

For more detailed information and analysis see the Company's Quarterly Report on Form 10-Q, which was filed with the Securities and Exchange Commission (SEC) on August 4, 2016.

Guidance

Based on Dicerna's current cash position and operating plan, the Company reiterates its expectation that it has sufficient cash to fund 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 second 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 49039068 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 August 4, 2016. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 49039068. The webcast will also be archived on the Company's website.

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, for other therapeutic areas in which the liver plays a key role, and for cancers that are genetically defined. 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.

About DCR-PH1

DCR-PH1 is being developed by Dicerna for the treatment of PH1 by addressing its pathology through the targeting and destruction of the messenger RNA (mRNA) produced by the HAO1 gene. HAO1 encodes glycolate oxidase (GO), an upstream enzyme involved in the production of oxalate, the mediator of pathogenesis and progression of PH1. In preclinical studies, DCR-PH1 inhibited HAO1 and increased levels of glycolate and reduced levels of urinary oxalate.

DCR-PH1 incorporates small interfering RNA (siRNA) formulated in a proprietary lipid nanoparticle (LNP) technology that is being investigated as a system for efficient delivery to the liver after intravenous (IV) administration. Dicerna obtained rights to this delivery technology through a licensing agreement with Arbutus Biopharma Corporation, formerly known as Tekmira Pharmaceuticals Corporation.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements, including, for example, 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.

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

	June 30, 2016	December 31, 2015
Cash and cash equivalents	$ 29,187	$ 56,058
Held-to-maturity investments	$ 40,003	$ 38,551
Total assets	$ 74,333	$ 100,023
Total liabilities	$ 9,365	$ 9,001
Total stockholders' equity	$ 64,968	$ 91,022

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

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2016	2015	2016	2015
Revenues	$ -	184	$ -	184
Operating expenses:				
Research and development	11,032	$ 11,875	22,296	$ 20,567
General and administrative	4,656	4,519	9,140	9,964
Total operating expenses	15,688	16,394	31,436	30,531
Loss from operations	(15,688)	(16,210)	(31,436)	(30,347)
Interest income	66	34	121	87
Net loss	$ (15,622)	$ (16,176)	$ (31,315)	$ (30,260)

Net loss per share - basic and diluted	$ (0.75)	$ (0.86)	$ (1.51)	$ (1.65)
Weighted average shares outstanding - basic and diluted	20,726,108	18,852,814	20,706,388	18,337,030

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Source: Dicerna Pharmaceuticals, Inc.