



November 2, 2017

Dicerna Reports Third Quarter 2017 Financial and Operating Results and Provides Corporate Update

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 ribonucleic acid interference (RNAi) therapeutics, today reported financial and operating results for the third quarter ended September 30, 2017.

"We are extremely pleased with the progress we made during the third quarter, and the fourth quarter is off to a great start, highlighted by the achievement of two key milestones for the Company," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "These include the submission of a Clinical Trial Application (CTA) in the United Kingdom for our lead program, DCR-PHXC, which we are developing for primary hyperoxaluria (PH), and the announcement of a strategic research collaboration with Boehringer Ingelheim International GmbH (BI), which, not only expands our GalXC™ pipeline to include a program for nonalcoholic steatohepatitis (NASH), but is reflective of the strength of our proprietary technology platform and our underlying intellectual property.

"As we approach year end and look ahead to 2018, we are working toward a number of other important catalysts for the Company. These include CTA submissions in additional European countries and an investigational new drug (IND) application filing in the U.S. for DCR-PHXC, as well as the commencement of our Phase 1 clinical trial of DCR-PHXC in the United Kingdom early in the first quarter of 2018. We also anticipate reporting human proof-of-concept data for this program in the second half of 2018. At the same time, we are focused on advancing our undisclosed GalXC program for a second rare disease, for which we plan to file an IND application and/or CTA in mid-2018, as well as our continued focus on DCR-HBVS for chronic hepatitis B virus, DCR-PCSK9 for hypercholesterolemia, and additional GalXC programs. Lastly, we are eager to embark on our new, joint GalXC collaboration with BI in chronic liver diseases, and we look forward to working closely with the company as we seek to discover and develop RNAi therapeutics to treat NASH."

GalXC™ Research Collaboration

On November 2, 2017, Dicerna announced a research collaboration and license agreement with BI to discover and develop novel GalXC RNAi therapeutics for the treatment of chronic liver diseases. The collaboration will initially focus on NASH, a devastating, chronic liver disease for which there is no approved treatment option. 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. Under the terms of the agreement, Dicerna may receive more than \$200 million from BI, including an upfront payment, development and commercial milestone payments, and research and development reimbursement for a GalXC candidate product addressing an undisclosed NASH target. Dicerna is also eligible to receive royalties tiered up to double-digits on worldwide net sales.

GalXC™ Pipeline Program Update

- 1 During the third quarter of 2017, Dicerna continued to progress preclinical activities for its three priority programs, which include DCR-PHXC for the treatment of PH, a program for an undisclosed rare disease, and DCR-HBVS for the treatment of chronic hepatitis B virus (HBV) infection. Dicerna's programs in clinical candidate selection include: DCR-PCSK9 for the treatment of hypercholesterolemia, and multiple programs targeting undisclosed targets in chronic liver diseases, cardiovascular diseases, and additional rare diseases.
 - 1 Primary Hyperoxaluria: On October 16, 2017, Dicerna announced it had submitted a CTA for DCR-PHXC to the Medicines and Healthcare products Regulatory Agency (MHRA) in the United Kingdom. DCR-PHXC is in development for the treatment of all types of PH. PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate that often results in kidney failure. Once the CTA is authorized by the MHRA and Ethics Committee approval has been obtained, Dicerna plans to conduct a Phase 1 trial as a randomized, single-blind, placebo-controlled, single-ascending dose study, enrolling up to 25 healthy volunteer subjects and up to 16 patients with PH type 1 (PH1) and PH type 2 (PH2). The primary objective of the study is to evaluate the safety and tolerability of single doses of DCR-PHXC, with participants being enrolled into as many as five sequential cohorts of increasing doses. Secondary endpoints include the pharmacokinetics of DCR-PHXC and its pharmacodynamic effects on oxalate biomarkers in plasma and urine. Dicerna plans to commence human clinical trials in the first quarter of 2018 and expects to have human proof of concept data in

the second half of 2018. Dicerna also plans to submit additional CTAs in other European countries later this year and plans to file an IND in the U.S. in the first quarter of 2018.

On July 15, 2017, in a series of presentations at the 12th International Workshop on Primary Hyperoxaluria for Professionals, Patients and Families in Tenerife, Spain, Dicerna presented new preclinical data suggesting the potential utility of DCR-PHXC for treating all forms of PH. In particular, Dicerna presented research from animal models demonstrating how DCR-PHXC inhibits the lactate dehydrogenase A (*LDHA*) gene, which Dicerna has identified as potentially being an optimal therapeutic target in patients with PH. *LDHA* inhibition was shown in animal models to reduce oxalate to normal or near-normal levels in PH1, PH2 and ethylene glycol-induced hyperoxaluria (a model for idiopathic PH). In animal studies, DCR-PHXC was shown to be well tolerated with no adverse effects in the liver.

During the workshop, Dicerna also reported interim data from its **Primary HYperoxaluria O**bservational **S**tudy (PHYOS), an international, multicenter, observational study in patients with a genetically confirmed diagnosis of PH1. PHYOS collected data on key biochemical parameters, including changes in oxalate, glycolate, and other metabolites, implicated in the pathogenesis of the disease. Twenty (20) patients were enrolled in the study with a median age at screening of 21 years (range 12-61 years). Over the six-month observation period, the variability (coefficient of variation) between 24-hour urine measurements of oxalate at different time points was 28%. Dicerna has now completed PHYOS and hopes to use the data to better understand the baseline PH1 disease state, which will help guide long-term DCR-PHXC drug development plans. These data will help the clinical team design future clinical studies using 24-hour urinary oxalate excretion as a surrogate marker for clinical benefits.

Undisclosed Rare Disease Involving the Liver: Dicerna advanced IND application-enabling activities for a second GalXC-based clinical candidate targeting an undisclosed rare disease. For competitive reasons, the Company has not yet publicly disclosed the target gene or disease. Dicerna is on track to file an IND application in the U.S. and/or CTA in Europe for this program in the second quarter of 2018.

Chronic Hepatitis B Virus (HBV): Dicerna declared a product candidate for DCR-HBVS, which targets HBV directly, and is conducting formal IND application-enabling work for this program. Current therapies for HBV rarely lead to a long-term functional cure as measured by the clearance of HBV surface antigen (HBsAg) and sustained HBV DNA suppression. Based on findings from its preclinical studies, Dicerna is evaluating whether its GalXC RNAi platform can produce an experimental HBV-targeted therapy that significantly reduces HBsAg expression in affected patients and that has the potential to be delivered in a subcutaneous dosing paradigm. The Company expects to file an IND application in the U.S. or CTA in Europe for this program approximately at the end of 2018.

Hypercholesterolemia: Dicerna continued to develop its DCR-PCSK9 program, which targets the PCSK9 gene for the treatment of hypercholesterolemia. The Company has selected a provisional clinical candidate for the program and is exploring ways to further optimize the program. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform has the potential to produce a PCSK9-targeted therapy with attractive commercial properties, such as small subcutaneous injection volumes and less frequent dosing.

Financial Condition and Operating Results

- 1 **Cash Position** - As of September 30, 2017, Dicerna had \$75.9 million in cash and cash equivalents and held-to-maturity investments, as compared to \$45.9 million as of December 31, 2016. In addition, the Company had \$1.1 million of restricted cash equivalents as of September 30, 2017, which reflects collateral securing the Company's operating lease obligation.
- 1 **Research and Development (R&D) Expenses** - R&D expenses were \$9.0 million and \$27.2 million for the three and nine months ended September 30, 2017, as compared to \$10.1 million and \$32.4 million for the same periods in 2016, respectively. The decrease in overall R&D expenses in both the three and nine month periods ended September 30, 2017, is primarily attributable to a reduction in employee-related expenses due to an overall decrease in headcount from the same periods in 2016, along with a decrease in non-cash stock-based compensation costs, as well as a substantial decrease in platform-related expenses and to a reduction in clinical manufacturing activities related to the Company's now discontinued DCR-PH1 and DCR-MYC programs, which were not based on Dicerna's GalXC platform. These decreases were partially offset by an increase in direct research and development expenses in both the three and nine month periods ended September 30, 2017, attributable to an overall increase in manufacturing activities and in toxicology study costs related to Dicerna's product candidates under its GalXC platform. Dicerna expects overall R&D expenses to increase during the fourth quarter of 2017, as compared to the third quarter of 2017, primarily as the Company ramps up clinical initiatives associated with DCR-PHXC.
- 1 **General and Administrative (G&A) Expenses** - G&A expenses were \$6.7 million and \$18.5 million for the three and nine months ended September 30, 2017, compared to \$4.3 million and \$13.5 million for the same periods in 2016, respectively. The increases are predominantly related to higher litigation costs as well as to higher salaries,

benefits and other employee-related expenses.

- 1 **Net Loss Attributable to Common Stockholders** - Net loss attributable to common stockholders was \$19.1 million and \$57.3 million for the three and nine months ended September 30, 2017, as compared to a net loss of \$14.2 million and \$45.5 million for the same periods in 2016, respectively. The overall increases in net loss attributable to common stockholders are due to the aforementioned changes in R&D and G&A expenses, as well as to the recording of non-cash dividends in 2017 on the Redeemable Convertible Preferred Stock.

For more detailed information and analysis, see Dicerna's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, which was filed with the Securities and Exchange Commission (SEC) on November 2, 2017.

Guidance

Dicerna believes that it has sufficient cash to fund the execution of its current clinical and operating plan into 2019, which includes focusing its resources on advancing its first three development programs into proof-of-concept clinical studies. This estimate assumes no new funding from additional collaboration agreements or from external financing events.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's third quarter 2017 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 95620574 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 approximately two hours after the completion of the call and will remain available for seven days. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 95620574. The webcast will also be archived on Dicerna's website.

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 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. 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 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 the Company believes will enable subcutaneous delivery of its 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, Dicerna's expected timeline and plans for development of DCR-PHXC and other pipeline programs, expectations related to the collaboration with BI, and guidance related to the anticipated availability of current liquidity. 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 risks relating to Dicerna's clinical and preclinical research and other risks identified under the heading "Risk Factors" included in the Company'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.

Dicerna Pharmaceuticals, Inc.
Consolidated Balance Sheet Information
(In thousands)

	September 30, 2017	December 31, 2016
Cash and cash equivalents	\$ 30,960	\$ 20,865
Held-to-maturity investments	\$ 44,959	\$ 25,009
Total assets	\$ 82,726	\$ 51,252
Total liabilities	\$ 10,522	\$ 10,044
Redeemable convertible preferred stock	\$ 75,983	\$ -
Total stockholders' (deficit) equity	\$ (3,779)	\$ 41,208

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2017	2016	2017	2016
Revenue	\$ 474	162	\$ 859	162
Operating expenses:				
Research and development	9,001	10,061	27,197	32,357
General and administrative	6,685	4,338	18,481	13,478
Total operating expenses	<u>15,686</u>	<u>14,399</u>	<u>45,678</u>	<u>45,835</u>
Loss from operations	(15,212)	(14,237)	(44,819)	(45,673)
Interest income	<u>179</u>	<u>61</u>	<u>360</u>	<u>182</u>
Net loss	\$ (15,033)	\$ (14,176)	\$ (44,459)	\$ (45,491)
Dividends on redeemable convertible preferred stock	(4,111)	-	(6,733)	-
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	<u>-</u>	<u>-</u>	<u>(6,144)</u>	<u>-</u>
Net loss attributable to common stockholders	\$ <u>(19,144)</u>	\$ <u>(14,176)</u>	\$ <u>(57,336)</u>	\$ <u>(45,491)</u>
Net loss per share - basic and diluted	\$ (0.92)	\$ (0.68)	\$ (2.76)	\$ (2.20)
Weighted average shares outstanding - basic and diluted	20,841,728	20,752,416	20,809,372	20,708,600

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