

Dicerna Reports Second 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 second quarter ended June 30, 2017.

"We are pleased to have reported a number of significant events during this quarter that support the execution of our business strategy," stated Douglas Fambrough, president and chief executive officer of Dicerna. "Specifically, the completion of our \$70 million convertible preferred stock financing and the key addition of Dr. Ralf Roskamp as chief medical officer, have served to further solidify our financial and leadership capacity. As a result, we are in a strong position to continue to advance DCR-PHXC, our lead GalXC™-based product candidate, into Phase 1 clinical studies for primary hyperoxaluria (PH) early next year and to continue to pursue Investigational New Drug (IND) application-enabling activities for our undisclosed rare disease program as well as for DCR-HBVS. More recently, during the 12th International Workshop on Primary Hyperoxaluria held this past July, we presented new preclinical data for DCR-PHXC demonstrating how inhibition of the lactate dehydrogenase A (*LDHA*) gene reduced oxalate production in multiple animal models of PH. These data highlight the role of *LDHA* as an optimal therapeutic target and the potential utility of DCR-PHXC to treat all forms of the disease. These findings indicate that this novel target may offer the ability to treat an expanded population of patients who currently have no other effective options."

GalXC™ Program Update

- | During the second quarter of 2017, Dicerna continued to progress preclinical activities for its four core therapeutic programs, including DCR-PHXC for PH, an undisclosed rare disease program, DCR-HBVS for hepatitis B virus, and DCR-PCSK9 for hypercholesterolemia. Dicerna's development activities are focused in the areas of rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases.
 - | Primary Hyperoxaluria: On July 15, 2017, in a series of presentations at the 12th International Workshop on Primary Hyperoxaluria, Dicerna presented new preclinical data showing DCR-PHXC's ability to inhibit *LDHA* resulting in consistent and significant reduction in urinary oxalate levels in animal models of PH type 1 (PH1), PH type 2 (PH2) and idiopathic PH. PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate that often results in kidney failure.

Research from multiple animal models of PH demonstrated how DCR-PHXC inhibits *LDHA*, which the Company has identified as potentially being an optimal therapeutic target in patients with the disease. The data highlights included:

- | *LDHA* inhibition reduces oxalate to normal or near-normal levels in PH types 1, 2, and ethylene glycol-induced hyperoxaluria (a model for idiopathic PH).
- | *LDHA* reduction has a near-linear correlation with oxalate reduction and offers a minimal metabolic intervention. These benefits of *LDHA* inhibition may translate into consistent therapeutic activity even in the event of a missed dose. There are numerous case reports of *LDHA* deficiency naturally occurring in healthy humans, with no reported adverse effects due to deficiency in the liver.
- | DCR-PHXC appeared to be well tolerated in these animal studies, with no adverse effects in the liver. Formal animal toxicology studies are ongoing.

During the workshop, Dicerna also reported data from its **P**rimarily **H**yperoxaluria **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, including changes in oxalate, glycolate, and other metabolites, implicated in the pathogenesis of the disease. Dicerna continues to advance PHYOS to facilitate DCR-PHXC development and hopes to use the data to better understand the baseline PH1 disease state, which will help guide long-term drug development plans.

- | Twenty (20) patients were enrolled in the study, with a median age at screening of 21 years (range 12-61 years). The patients had been diagnosed at a median age of 7 years (range 1-59 years), and 14 patients (74%) had a medical history of renal stones.

- Over the six-month observation period, the variability (coefficient of variation) between 24-hour urine measurements of oxalate at different time points was 28%.
- These data will be used by Dicerna's clinical team in the design of future clinical studies using 24-hour urinary oxalate excretion as a surrogate marker for clinical benefit.

Dicerna is on track to file a clinical trial application (CTA) in Europe for DCR-PHXC in late 2017 and to commence human clinical trials in the first quarter of 2018. During the workshop, Dicerna disclosed that the DCR-PHXC clinical trial will be conducted at multiple sites in Europe and will include both healthy volunteer and patient cohorts. The Company anticipates that study participants will receive a single ascending dose of DCR-PHXC via subcutaneous injection, transitioning, as appropriate, to multiple ascending doses. The primary endpoints will include safety and tolerability, urine and plasma biomarkers, and pharmacokinetics.

- 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 continued to progress its DCR-HBVS program, which targets HBV directly, and has initiated formal IND-enabling activities. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg) and sustained HBV deoxyribonucleic acid 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 at approximately the end of 2018.
- Hypercholesterolemia:** Dicerna continued to develop its DCR-PCSK9 program, which targets the PCSK9 gene and will be evaluated for the treatment of statin-refractory patients with hypercholesterolemia. The Company is positioned to advance DCR-PCSK9 into formal preclinical development. 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.

Financing Update

- As previously reported, on April 11, 2017, Dicerna closed a stock purchase transaction for the sale of redeemable convertible preferred stock (Preferred Stock) to a syndicate of current and new investors led by Bain Capital Life Sciences, under which the Company received gross proceeds of \$70.0 million (Private Placement). At the closing, Dicerna issued 700,000 shares of Preferred Stock, which are convertible into common shares at an initial conversion price of \$3.19 per share. In addition to the lead investor, other participants in the Private Placement included Cormorant Asset Management, Domain Associates, EcoR1 Capital, RA Capital and Skyline Ventures, among others. Under the terms of the Preferred Stock purchase agreement, Adam M. Koppel, M.D., Ph.D., a managing director of Bain Capital Life Sciences, joined Dicerna's board of directors, which has been expanded to nine seats.

Corporate Update

- On June 8, 2017, Dicerna announced the appointment of industry veteran Ralf Roskamp, M.D., as chief medical officer. Dr. Roskamp brings to Dicerna more than 20 years of research and development experience spanning the entire drug development cycle, from preclinical through product commercialization. He has been responsible for numerous IND applications, the design and execution of clinical development programs, and new drug applications across multiple therapeutic areas including diabetes, cardiovascular, respiratory, and orphan drugs. Approved products for which Dr. Roskamp was involved include Natpara®, Amaryl®, Lantus®, Apidra® and Simcor®. Dr. Roskamp is a pediatric endocrinologist and received his M.D. from the University of Bonn, Germany.

Financial Condition and Operating Results

- Cash Position** - As of June 30, 2017, Dicerna had \$88.7 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 June 30, 2017, which reflects collateral securing the Company's operating lease obligation. The increase in cash and cash equivalents and held-to-maturity investments was due chiefly to the addition of funds generated by the Company's \$70 million Private Placement, which closed on April 11, 2017.
- Research and Development (R&D) Expenses** - R&D expenses were \$9.3 million and \$18.2 million for the three and six months ended June 30, 2017, as compared to \$11.0 million and \$22.3 million for the same periods in 2016, respectively. The decrease was due primarily to a reduction in platform-related expenses, resulting from the timing of

activities related to discovery and early development programs, including supply and external study costs; to a decrease in employee-related expenses, including non-cash stock-based compensation costs; and to a reduction in clinical and manufacturing activities related to the Company's now discontinued DCR-PH1 and DCR-MYC programs, both of which Dicerna anticipates will be fully wound down before the end of 2017. These decreases were partially offset by an increase in direct R&D expenses due to an overall increase in manufacturing activities and in toxicology study costs related to Dicerna's new candidates under its GalXC platform.

- ▮ **General and Administrative (G&A) Expenses** - G&A expenses were \$6.3 million and \$11.8 million for the three and six months ended June 30, 2017, compared to \$4.7 million and \$9.1 million for the same periods in 2016, respectively. The increase was predominantly related to higher litigation-related expenses as well as to higher salaries, benefits and other employee-related expenses.
- ▮ **Net Loss Attributable to Common Stockholders** - Net loss attributable to common stockholders was \$24.0 million and \$38.2 million for the three and six months ended June 30, 2017, as compared to a net loss of \$15.6 million and \$31.3 million for the same periods in 2016, respectively. In addition to the aforementioned changes in R&D and G&A expenses, net loss attributable to common stockholders also increased as a result of the recording of Preferred Stock dividends, which include a one-time non-cash deemed dividend charge of \$6.1 million.

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

Guidance

With the closing of its Preferred Stock transaction, 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 and a fourth program into formal preclinical development. This estimate assumes no additional funding from new collaboration agreements or from additional financing events.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's second 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 38183667 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 10, 2017. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 38183667. 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, our expected timeline and plans for development of DCR-PHXC and other pipeline programs, 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 risks 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. Consolidated Balance Sheet Information (In thousands)

	June 30, 2017	December 31, 2016
Cash and cash equivalents	\$ 38,777	\$ 20,865
Held-to-maturity investments	\$ 49,953	\$ 25,009
Total assets	\$ 94,723	\$ 51,252
Total liabilities	\$ 9,583	\$ 10,044
Redeemable convertible preferred stock	\$ 71,872	\$ -
Total stockholders' equity	\$ 13,268	\$ 41,208

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

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2017	2016	2017	2016
Revenue	\$ 252	-	\$ 385	-
Operating expenses:				
Research and development	9,320	11,032	18,196	22,296
General and administrative	6,300	4,656	11,796	9,140
Total operating expenses	15,620	15,688	29,992	31,436
Loss from operations	(15,368)	(15,688)	(29,607)	(31,436)
Interest income	143	66	181	121
Net loss	\$ (15,225)	\$ (15,622)	\$ (29,426)	\$ (31,315)
Dividends on redeemable convertible preferred stock	(2,622)	-	(2,622)	-
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	(6,144)	-	(6,144)	-
Net loss attributable to common stockholders	\$ (23,991)	\$ (15,622)	\$ (38,192)	\$ (31,315)
Net loss per share - basic and diluted	\$ (1.15)	\$ (0.75)	\$ (1.84)	\$ (1.51)
Weighted average shares outstanding - basic and diluted	20,794,193	20,726,108	20,792,925	20,706,388

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Investors:

Rx Communications Group
Paula Schwartz, 917-322-2216
pschwartz@rxir.com

or

Media:

SmithSolve
Alex Van Rees, 973-442-1555 ext. 111
alex.vanrees@smithsolve.com

Source: Dicerna Pharmaceuticals, Inc.

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