Dicerna Reports First Quarter 2017 Financial and Operational Results and Provides Corporate Update

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

CAMBRIDGE, Mass., May 8, 2017 -- Dicerna Pharmaceuticals, Inc. (NASDAQ: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operational results for the first quarter ended March 31, 2017.

"While continuing to focus on our research and development activities during the first quarter, we announced a major $70.0 million convertible preferred stock financing, which subsequently closed on April 11, 2017," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "The financing, led by Bain Capital Life Sciences and a syndicate of current and new investors, lends an additional level of validation to the potential of our proprietary GalXC™ RNAi technology platform. Furthermore, the funds, when combined with cash already on-hand, provide us with the necessary resources to execute our stated strategy, which includes pursuing the advancement of our core therapeutic programs. Specifically, as we look forward from today and into 2019, we expect to be able to advance our first three development programs, including DCR-PHXC, a second undisclosed rare disease program and DCR-HBV, into proof of concept studies, while progressing DCR-PCSK9 toward formal preclinical development, resulting in a number of key, value-creating inflection points. In the nearer term, I look forward to presenting new preclinical data for DCR-PHXC later this year and to our expected Investigational New Drug (IND) or Clinical Trial Application (CTA) filing at the end of 2017."

GalXC™ Program Update

- During the first quarter of 2017, Dicerna continued to advance its preclinical pipeline of liver-targeted GalXC product candidates. These long-acting, subcutaneously delivered therapeutic candidates are within the Company's strategic focus areas of rare diseases, chronic liver diseases, cardiovascular diseases and viral infectious diseases. Dicerna has prioritized four therapeutic programs that the Company believes have favorable probabilities of success, including DCR-PHXC, an undisclosed rare disease program, DCR-HBV and DCR-PCSK9.

    - Primary Hyperoxaluria Type 1 (PH1): Dicerna continued to progress IND-enabling studies for its lead product candidate, DCR-PHXC, which is being evaluated for the treatment of patients with PH1. PH1 is a rare, inborn error of metabolism in which the liver produces excessive levels of oxalate, which in turn causes damage to the kidneys and other tissues in the body. Dicerna plans to announce additional details about its DCR-PHXC program at the Oxalosis and Hyperoxaluria Foundation's 12th International Workshop on Primary Hyperoxaluria in Tenerife, Spain, in July 2017, and is on track to file an IND submission or CTA in late 2017.

The Company plans to commence Phase 1 clinical trials in the first quarter of 2018.

- Undisclosed Rare Disease Involving the Liver: During the period, Dicerna initiated IND-enabling activities for a second GalXC clinical candidate targeting an undisclosed rare disease. The Company expects to file an IND application or CTA for this program in the second quarter of 2018.

- Chronic Hepatitis B Virus (HBV): Dicerna has recently initiated formal IND-enabling studies for DCR-HBV, which targets HBV directly. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg). 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 or CTA for this program around the end of 2018.

- Cardiovascular Disease: 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 properties such as small subcutaneous injection volumes and relatively infrequent dosing.

Financial Condition and Operating Results

- **Cash Position** – As of March 31, 2017, Dicerna had $32.9 million in cash and cash equivalents and held-to-maturity investments, as compared to $45.9 million in cash and cash equivalents and held-to-maturity investments as of December 31, 2016. In addition, the Company had $1.1 million of restricted cash equivalents as of March 31, 2017, which reflects collateral securing the Company's operating lease obligation.

- **Research and Development (R&D) Expenses** – R&D expenses for the first quarter of 2017 were $8.9 million, as compared to $11.3 million for the same period in 2016. The decrease was due primarily to a reduction in clinical and manufacturing activities related to the Company's now discontinued PH1 and MYC programs, both of which will be fully wound down before the end of the current year, offset by an overall increase in manufacturing activities related to new candidates under the GalXC platform; a decrease in platform-related expenses; and a decrease in employee-related expenses,

due largely to an overall decrease in headcount from the prior year and slightly lower stock-based compensation costs.

- **General and Administrative (G&A) Expenses** – G&A expenses for the first quarter of 2017 were $5.5 million, compared to $4.5 million for the same period in 2016. The increase was primarily attributable to higher legal costs, partially offset by a slight reduction in stock-based compensation costs.

- **Net Loss** – Net loss for the first quarter of 2017 was $14.2 million compared to a net loss of $15.7 million for the same period in 2016.

- **Financing** – On March 30, 2017, Dicerna entered into a stock purchase agreement with a syndicate of current and new investors, led by Bain Capital Life Sciences, for the sale of 700,000 shares of redeemable convertible preferred stock (Preferred Stock) at a purchase price of $100.00 per share, for total gross proceeds of $70.0 million. Participants in the financing include Cormorant Asset Management, Domain Associates, EcoR1 Capital, RA Capital and Skyline Ventures, among others. The Preferred Stock is convertible into common shares at a conversion price of $3.19 per share.

For more detailed information and analysis, see Dicerna's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, which was filed with the Securities and Exchange Commission (SEC) on May 8, 2017.

Subsequent Event

- On April 11, 2017, Dicerna announced that it closed its stock purchase transaction for the sale of redeemable convertible preferred stock, under which the Company received gross proceeds of $70.0 million. Under 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.

Guidance

With the closing of its Preferred Stock transaction, Dicerna believes that it has sufficient cash to fund the execution of its current operating plan into 2019, which includes focusing its resources on advancing its first three development programs into proof of concept studies and a fourth program into formal preclinical development. This estimate assumes no additional funding from new partnership agreements or from additional financing events.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review the Company's first 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 7812180 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 May 8, 2017. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 7812180. 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 RNAi-based therapeutics for diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company 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. The Company 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 (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 anticipated use of proceeds from the Preferred Stock transaction, 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 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)

	March 31, 2017	December 31, 2016
Cash and cash equivalents	$ 22,873	$ 20,865
Held-to-maturity investments	$ 10,002	$ 25,009
Total assets	$ 39,436	$ 51,252
Total liabilities	$ 10,347	$ 10,044
Total stockholders' equity	$ 29,089	$ 41,208

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

	For the Three Months Ended March 31,	
	2017	2016
Revenue	$ 133	$ -
Operating expenses:		
Research and development	8,876	11,264
General and administrative	5,496	4,484
Total operating expenses	14,372	15,748
Loss from operations	(14,239)	(15,748)
Interest income	38	55
Net loss	$ (14,201)	$ (15,693)
Net loss per share - basic and diluted	$ (0.68)	$ (0.76)
Weighted average shares outstanding - basic and diluted	20,791,644	20,646,793

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