Dicerna Reports First Quarter 2018 Financial and Operating Results and Provides Corporate Update

May 14, 2018

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 14, 2018-- Dicerna Pharmaceuticals, Inc. (NASDAQ: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operating results for the first quarter ended March 31, 2018.

“Dicerna is off to a strong start in 2018 with the achievement of a number of important milestones for our lead GalXC™ program, DCR-PHXC, currently in development for primary hyperoxaluria,” said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. “With the healthy volunteer phase of the PHYOX Phase 1 study now complete, we have initiated the second phase of the trial, and dosing of the first primary hyperoxaluria patient with DCR-PHXC is imminent. While the PHYOX study remains blinded to treatment assignment, the early safety and tolerability data in healthy volunteers are very encouraging. The FDA’s acceptance of our investigational new drug application (IND) for DCR-PHXC gives us the greenlight to expand the trial into the U.S. We expect 2018 to be an eventful year for Dicerna as we hope to achieve clinical proof-of-concept results from this multi-center study in the second half of 2018. Additionally, we look forward to achieving key milestones for our other priority programs, DCR-HBVS for chronic hepatitis B virus and an undisclosed program for a rare disease of the liver, including the filing of regulatory clearances to initiate clinical trials later this year.

“On an important note, we recently announced that we have resolved all ongoing litigation between Dicerna and Alnylam Pharmaceuticals. This settlement allows us to advance all of our existing and currently anticipated pipeline programs while maintaining a strong balance sheet. With the litigation now behind us, we are able to focus all of our resources on progressing our clinical programs, discovery programs, and partnering initiatives in accordance with our strategy and timelines. In a separate disclosure, we reported that Bruce Peacock, a member of the Board of Directors, will be stepping down from his board position at Dicerna’s Annual Meeting of Stockholders in June. I would like to thank Bruce for his service and wise counsel to the Company over the past four years and wish him well in his future endeavors.”

GalXC™ Pipeline Update

- GalXC Priority Programs: During the first quarter of 2018, Dicerna continued to actively progress the development of its three priority programs and remains on track to advance these programs into clinical development by early 2019.
    - Primary Hyperoxaluria (PH): During the first quarter of 2018, Dicerna advanced its PHYOX Phase 1 clinical trial of DCR-PHXC in healthy volunteers and patients with PH. DCR-PHXC is the only potential treatment in development for all forms of the disease. PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate that often results in kidney failure.
        - Dicerna has now completed dosing of all normal healthy volunteers (NHVs) in the Group A portion of the PHYOX study and dosing of the first PH patient with DCR-PHXC in the Group B portion of the study is imminent. While the study remains blinded to treatment assignment, topline results from Group A show there were no serious adverse events (SAEs) and no discontinuations. There have been two mild-to-moderate transient injection site reactions at doses of 6 and 12 mg/kg involving erythema and tenderness, lasting no more than 36 hours.
        - The PHYOX trial is a Phase 1 single-ascending dose study of DCR-PHXC in NHVs and patients with PH. The study is divided into two groups: Group A is a placebo-controlled, single-blind, single-center study that has enrolled 25 NHVs; Group B is an open-label, multi-center study enrolling up to 16 patients with primary hyperoxaluria type 1 (PH1) and type 2 (PH2). The primary objective of the study is to evaluate the safety and tolerability of single doses of DCR-PHXC in both groups. The secondary objectives are to characterize the pharmacokinetics of single doses of DCR-PHXC in NHVs and patients with PH, and to evaluate the pharmacodynamic effect of single doses of DCR-PHXC on biochemical markers, including but not limited to, changes in urine oxalate concentrations.
        - On March 30, 2018, Dicerna received a notice from the U.S. Food and Drug Administration (FDA) indicating the acceptance of its IND to conduct the DCR-PHXC Phase 1 study in the U.S. In addition to this active IND, the Company has active CTAs in the United Kingdom, France and Germany, having received the appropriate regulatory and ethical approvals for the trial in these countries. A CTA has been submitted and is pending approval in the Netherlands.
        - Dicerna is on track to have clinical proof-of-concept (POC) data from the PHYOX trial in the second half of 2018.
        - Dicerna expects to initiate a multi-dose Phase 2/3 study of DCR-PHXC in the first quarter of 2019, pending positive POC data and regulatory feedback.
    - Chronic Hepatitis B Virus (HBV): Dicerna expects to file regulatory clearances to initiate a clinical trial for its DCR-HBVS program in development for chronic HBV, during the fourth quarter of 2018, and expects to begin clinical studies shortly thereafter.
    - Undisclosed Rare Disease Involving the Liver: Dicerna continued to prepare for its regulatory filing for its second GalXC-based clinical candidate targeting a liver expressed gene involved in a serious rare disease. The Company is seeking a risk-sharing collaborator for this program before it files regulatory clearances to initiate a clinical trial, likely in the second half of 2018. For competitive reasons, the Company has not yet publicly disclosed the target gene or disease.
- NASH Collaboration with Boehringer Ingelheim (BI): During the first quarter of 2018, Dicerna continued to successfully execute on the program in accordance with the work plan for this collaboration. The collaboration is focused on chronic liver diseases, with an initial focus on nonalcoholic steatohepatitis (NASH).
- GalXC Platform Improvement: During the first quarter of 2018, Dicerna continued to optimize its GalXC technology platform, which has enabled the development of next generation GalXC molecules that can be applied to any target gene. Next

generation GalXC improvements have yielded a longer duration of action and higher potency of target gene silencing against multiple targets in animal models. Dicerna anticipates utilizing its next generation GalXC molecules in its DCR-PCSK9 program for the treatment of hypercholesterolemia as well as in additional programs.

Conclusion of Litigation

- On April 18, 2018, Dicerna and Alynlam entered into a Confidential Settlement Agreement and General Release (the Settlement Agreement), resolving all ongoing litigation between the two companies. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the litigation between Dicerna and Alnylam. Dicerna denies wrongdoing and did not admit wrongdoing as part of the Settlement Agreement. Dicerna has agreed to make the following payments to Alnylam: (i) a $2.0 million upfront payment in cash; (ii) an additional $13.0 million in cash paid over the next four years, to be paid as 10% of any upfront or first year cash consideration that the Company receives pursuant to future GalXC technology-based collaborations (excluding any amounts received or to be received by the Company from its existing collaboration with BI); (iii) issuance of 983,208 shares of Dicerna common stock. The Settlement Agreement does not include any licenses to any intellectual property from either party and does not include any royalties or milestones related to product development. For additional detail, please see Dicerna's Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, which was filed with the Securities and Exchange Commission (SEC) on May 14, 2018.

Financial Condition and Operating Results

- **Cash Position** – As of March 31, 2018, Dicerna had $97.8 million in cash and cash equivalents and held-to-maturity investments, as compared to $113.7 million in cash and cash equivalents and held-to-maturity investments as of December 31, 2017. Additionally, the Company had $0.7 million of restricted cash equivalents as of March 31, 2018, which reflects collateral securing the Company's operating lease obligation.
- **Revenue** – For the three-month period ended March 31, 2018, Dicerna recognized $1.5 million of revenue associated with the BI Agreement. This amount primarily represents partial amortization of the $10.0 million non-refundable upfront payment from BI, as well as certain reimbursable third-party research expenses which are billable to BI. Dicerna expects to recognize the remainder of the initial transaction price on a straight-line basis through June 30, 2019. Dicerna does not expect to generate any product revenue for the foreseeable future.
- **Research and Development (R&D) Expenses** – R&D expenses for the first quarter of 2018 were $9.9 million, as compared to $8.7 million for the same quarter in 2017. The increase was predominantly due to higher direct research and development and employee-related expenses, partially offset by lower platform-related expenses.
- **General and Administrative (G&A) Expenses** – G&A expenses for the first quarter of 2018 were $7.5 million, as compared to $5.5 million for the same quarter in 2017. The increase was predominantly related to higher legal costs associated with the litigation with Alnylam, and to higher corporate legal expenses, partially offset by lower consulting expenses. We expect our G&A expenses to increase significantly in the second quarter of 2018, as compared to the three-month period ended March 31, 2018, as a result of the one-time charges associated with the Settlement Agreement.
- **Net Loss** – Net loss was $15.6 million for the first quarter of 2018, as compared to a net loss of $14.2 million for the same quarter in 2017. This increase is attributable to higher operating expenses, offset by higher revenues and interest income.

For more detailed information and analysis, see Dicerna's Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, which was filed with the Securities and Exchange Commission (SEC) on May 14, 2018.

Guidance

Dicerna believes that it has sufficient cash to fund the execution of its current clinical and operating plan through 2019, which includes focusing its resources on advancing its DCR-PHXC development program through proof-of-concept trials and into advanced clinical development, and advancing its DCR-HBVS development program into proof-of-concept studies in HBV patients. 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 first quarter 2018 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 9666285 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 9666285. 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, viral infectious diseases, chronic liver diseases, and cardiovascular 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 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, viral infectious diseases,

chronic liver diseases, and cardiovascular diseases. Using GalXC, Dicerna scientists attach N-acetylgalactosamine sugars directly to the extended region of the Company's proprietary RNAi 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 seen in preclinical studies 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 duration and usage of current cash and cash equivalents. 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)

	March 31, 2018	December 31, 2017
Cash and cash equivalents	$ 43,046	$ 68,789
Held-to-maturity investments	$ 54,770	$ 44,889
Total assets	$ 104,074	$ 121,002
Total liabilities	$ 16,188	$ 19,916
Total stockholders' equity	$ 87,886	$ 101,086

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

	For the Three Months Ended March 31,	
	2018	**2017**
Revenue	$ 1,545	-
Operating expenses:		
Research and development	9,893	8,743
General and administrative	7,519	5,496
Total operating expenses	17,412	14,239
Loss from operations	(15,867)	(14,239)
Interest income	288	38
Net loss	$ (15,579)	$ (14,201)
Net loss per share - basic and diluted	$ (0.30)	$ (0.68)
Weighted average shares outstanding - basic and diluted	51,723,349	20,791,644

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Source: Dicerna Pharmaceuticals, Inc.

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