March 8, 2018

Dicerna Reports Fourth Quarter and Year End 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 fourth quarter and full-year ended December 31, 2017.

"2017 was a transformative year for Dicerna, as we successfully transitioned to a clinical stage company advancing our growing pipeline of GalXC™-based RNAi therapeutics, and signed our first GalXC platform collaboration agreement with Boehringer Ingelheim," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "With our lead candidate, DCR-PHXC for the treatment of primary hyperoxaluria (PH), now in a Phase 1 clinical trial, and our other priority programs, including DCR-HBVS for chronic hepatitis B and an undisclosed program for a rare disease of the liver, following quickly behind, we plan to have three GalXC product candidates in clinical development by early 2019.

"We entered this year with a significantly stronger balance sheet and simplified capital structure, which we anticipate permits the funding of our development activities through 2019. Subsequent to our recent $46.0 million follow-on offering of common stock, at December 31, 2017, we had $113.7 million in cash and cash equivalents, which we expect will be sufficient to advance our DCR-PHXC development program through proof-of-concept trials and into advanced clinical development and advance our DCR-HBVS development program into clinical proof-of-concept studies in HBV patients, and provides a runway for us to further develop our earlier stage pipeline programs. As we advance through 2018, we are focused on seeking to achieve the key clinical milestones ahead of us, including dosing the first PH patient in our Phase 1 trial, the submission of clinical trial applications (CTAs) and/or investigational new drug (IND) applications for our other priority programs, and sharing clinical proof-of-concept data from our DCR-PHXC Phase 1 study in the second half of 2018."

GalXC™ Research Collaboration

- On October 27, 2017, Dicerna entered into a research collaboration and license agreement with Boehringer Ingelheim (BI) to discover and develop novel GalXC™ RNAi therapeutics for the treatment of chronic liver diseases. The collaboration initially focuses on nonalcoholic steatohepatitis (NASH), a chronic liver disease for which there is no approved treatment option. The BI agreement is for the development of product candidates against one target gene with an option for BI to add the development of product candidates against a second target gene. Dicerna granted BI a worldwide license to the product candidates in connection with the agreement. Under the terms of the agreement, BI agreed to pay Dicerna a non-refundable upfront payment of $10.0 million for the first target. During the term of the research program, BI will reimburse Dicerna the cost of materials and third-party expenses that have been included in the preclinical studies up to an agreed-upon limit. Dicerna is eligible to receive up to $191.0 million in potential development and commercial milestones, as well as royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double-digits. BI's option to add a second target would provide for an option fee payment and success-based development and commercialization milestones and royalty payments to Dicerna.

GalXC™ Pipeline Program Update

- During the fourth quarter of 2017, Dicerna continued to progress development activities for its three priority programs, which include DCR-PHXC for the treatment of PH, DCR-HBVS for the treatment of chronic hepatitis B virus (HBV) infection, and a program for an undisclosed rare disease involving the liver.

  Additionally, the Company further optimized its GalXC™ technology platform, enabling the development of next generation GalXC molecules that can be applied to any target gene and program. Improvements to Dicerna's GalXC compounds include modification of the tetraloop end of the molecule resulting in a substantially longer duration of action and improved potency in animal models. These modifications are unique to Dicerna's GalXC technology platform and are not gene-specific, so they are easily applied to GalXC molecules targeting any gene. Dicerna plans to utilize its next generation GalXC molecules in its DCR-PCSK9 program for the treatment of hypercholesterolemia, and in additional programs targeting chronic liver diseases, cardiovascular diseases, and additional rare diseases.

  - Primary Hyperoxaluria: 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.

- 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.
- On December 7, 2017, the Company announced it dosed the first healthy volunteer in a Phase 1 clinical trial of DCR-PHXC, named PHYOX. PHYOX is a single-ascending dose study of DCR-PHXC in normal healthy volunteers (NHVs) and patients with PH. The study is divided into two groups: Group A is a placebo-controlled, single-blind, single-center study enrolling up to 25 NHVs; Group B is an open-label, multi-center study enrolling up to 16 patients with PH types 1 (PH1) and 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 and its pharmacodynamic effect on biochemical markers, including changes in urine oxalate concentrations.
- Dicerna has submitted CTAs for the PHYOX study in Germany, France and the Netherlands, and intends to submit additional CTAs in other European countries later this year.
- Dicerna plans to dose the first patient with PH in the second quarter of 2018, and expects to have clinical proof-of-concept (POC) data from the PHYOX study 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 approvals.

- Chronic Hepatitis B Virus: Dicerna has declared a GalXC-based product candidate, DCR-HBVS, which targets HBV directly, and is conducting formal non-clinical development studies. The Company expects to file an IND in the U.S. or CTA during the fourth quarter of 2018.

- Undisclosed Rare Disease Involving the Liver: Dicerna advanced IND application-enabling activities for a second GalXC-based clinical candidate targeting a liver expressed gene involved in a serious rare disease. For competitive reasons, the Company has not yet publicly disclosed the target gene or disease. Dicerna plans to seek a risk-sharing collaborator for this program before it files an IND in the U.S. and/or CTA in Europe.

Financing Update

- On December 18, 2017, Dicerna closed a follow-on public offering of 6,571,428 shares of its common stock with aggregate gross proceeds totaling $46.0 million. In connection with the offering, Dicerna entered into a Letter Agreement with the holders of all of the outstanding shares of the Company's Redeemable Convertible Preferred Stock (RCPS), resulting in the conversion of the RCPS into an aggregate of approximately 24.2 million shares of the Company's common stock at the completion of the follow-on public offering. As a result, Dicerna's capital structure is now comprised of approximately 51.6 million shares of all common stock, and no RCPS shares remain outstanding.

Financial Condition and Operating Results

- **Cash Position** - As of December 31, 2017, Dicerna had $113.7 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. Additionally, the Company had $0.7 million of restricted cash equivalents as of December 31, 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 primarily to the addition of funds generated by the Company's $70.0 million Private Placement, which closed on April 11, 2017, the $46.0 million follow-on public offering of shares of its common stock, which closed on December 18, 2017, and to the upfront payment received from BI.
- **Revenue** - As of December 31, 2017, Dicerna recognized $1.2 million of revenue associated with the BI Agreement. This amount represents partial amortization of the $10.0 million upfront payment received from BI, as well as reimbursable third-party research expenses which are billable to BI. Dicerna currently expects to recognize the remaining $9.0 million of the aforementioned non-refundable upfront payment 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 fourth quarter of 2017 were $9.8 million, as compared to $9.3 million for the same quarter in 2016. The increase was due to higher direct research and development expenses, including drug substance, toxicology study and manufacturing activities associated with the Company's GalXC platform product candidates, partially offset by a decrease in platform-related expenses, which decreased primarily due to lower spending in discovery and early development programs, and by a decrease in employee-related expenses.

  Total R&D expenses for the year ended December 31, 2017, decreased by $4.7 million, as compared to the same period in 2016, despite an overall increase in direct research and development expenses, which was due to higher

development and manufacturing activities associated with Dicerna's GalXC platform product candidates, partially offset by a decrease in comparative clinical activities related to the Company's non-GalXC platform clinical trials, which were discontinued during 2016. Platform-related expenses decreased primarily as a result of lower spending in discovery and early development programs, which advanced in 2017 into manufacturing and clinical testing. Employee-related expenses decreased due to an overall decrease in headcount from 2016, along with a decrease in non-cash stock-based compensation costs.

Dicerna expects its overall R&D expenses to increase in 2018, as compared to 2017, as the Company continues spending on its development programs and related resources, including the continued advancement of its lead product candidate, DCR-PHXC, through clinical trials.

- **General and Administrative (G&A) Expenses** - G&A expenses for the fourth quarter of 2017 were $7.4 million, as compared to $4.9 million for the same quarter in 2016. The increase was largely due to higher legal costs, most notably those incurred in connection with litigation.

  Total G&A expenses were $25.9 million and $18.3 million for the years ended December 31, 2017 and 2016, respectively. The increase was primarily due to higher litigation costs, in addition to higher salaries, benefits and other employee-related expenses. Dicerna expects G&A expenses to decrease in 2018, as compared to 2017, largely as the Company expects to incur lower legal expenses.

- **Net Loss Attributable to Common Stockholders** - Net loss attributable to common stockholders was $22.8 million for the fourth quarter of 2017, as compared to a net loss of $14.0 million for the same quarter in 2016. The overall increase in net loss attributable to common stockholders was due to higher R&D and G&A expenses incurred during the fourth quarter of 2017 as compared to the same period in 2016, as well as to the recording, in 2017, of $7.2 million of non-cash dividends and deemed dividends related to and upon conversion of the Company's RCPS.

  Total net loss attributable to common stockholders was $80.1 million and $59.5 million for the years ended December 31, 2017 and 2016, respectively. The overall increase in net loss attributable to common stockholders was due to the recording, in 2017, of $20.1 million of non-cash dividends and deemed dividends related to and upon the conversion of the RCPS, as well as to higher G&A expenses, partially offset by higher collaboration and grant revenues and lower R&D expenses.

For more detailed information and analysis, see Dicerna's Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the Securities and Exchange Commission (SEC) on March 8, 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 fourth quarter and year end 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 9997829 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 9997829. 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, chronic liver diseases, cardiovascular disease and viral infectious 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 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-K 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)

	December 31, 2017	December 31, 2016
Cash and cash equivalents	$ 68,789	$ 20,865
Held-to-maturity investments	$ 44,889	$ 25,009
Total assets	$ 120,884	$ 51,252
Total liabilities	$ 19,646	$ 10,044
Total stockholders' equity	$ 101,238	$ 41,208

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

	For the Three Months Ended December 31,		For the Year Ended December 31,	
	2017	2016	2017	2016
Revenue	$ 1,418	133	$ 2,277	295
Operating expenses:				
Research and development	9,786	9,337	36,983	41,694
General and administrative	7,400	4,871	25,881	18,349
Total operating expenses	17,186	14,208	62,864	60,043

Loss from operations	(15,768)	(14,075)	(60,587)	(59,748)
Interest income	179	53	539	235
Net loss	$ (15,589)	$ (14,022)	$ (60,048)	$ (59,513)
Dividends on redeemable convertible preferred stock	(3,378)	-	(10,111)	-
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	-	-	(6,144)	-
Deemed dividend on conversion of redeemable convertible preferred stock	(3,837)	-	(3,837)	-
Net loss attributable to common stockholders	$ (22,804)	$ (14,022)	$ (80,140)	$ (59,513)
Net loss per share - basic and diluted	$ (0.90)	$ (0.68)	$ (3.66)	$ (2.87)
Weighted average shares outstanding - basic and diluted	25,205,415	20,753,001	21,917,415	20,719,761

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Investor Contact:
Rx Communications Group
Paula Schwartz, 917-322-2216
pschwartz@rxir.com
or
Media Contact:
SmithSolve
Alex Van Rees, 973-442-1555 ext. 111
alex.vanrees@smithsolve.com

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