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

August 8, 2018

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 8, 2018-- 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, 2018.

"During the second quarter and subsequent period, we took a significant step forward in the development of our lead program, DCR-PHXC, as well as our broad pipeline of novel GalXC™-based therapeutics," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Our PHYOX Phase 1 trial of DCR-PHXC for primary hyperoxaluria is progressing efficiently, with 10 of 16 PH type 1 (PH1) and PH type 2 (PH2) patients in the Group B portion of the trial now dosed, and we expect to report interim results later in the third quarter and publicly present trial results in the fourth quarter of 2018. We continue to expect that we will initiate a pivotal registration trial of DCR-PHXC in the first quarter of 2019. In parallel, we continued to advance our development program for DCR-HBVS for the treatment of chronic hepatitis B virus toward clinical trials and in the fourth quarter intend to file regulatory clearances that, if granted, will permit us to commence human clinical trials for DCR-HBVS. Finally, with the Alnylam litigation now behind us, we have seen a notable increase in active discussions with potential partners and collaborators. With these advancements, we remain on track to have three programs in clinical trials in the first half of 2019 and expect to achieve other development milestones in the coming months."

GalXC™ Pipeline Update

- Primary Hyperoxaluria (PH): Dicerna continued to advance the PHYOX Phase 1 clinical trial of DCR-PHXC, which is in development to treat all forms of PH. PHYOX is a single-ascending dose study of DCR-PHXC in normal healthy volunteers (NHVs) and patients with PH. PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate that often results in kidney failure.
    - In May 2018, Dicerna dosed the first PH patient with DCR-PHXC in the Group B portion of the PHYOX study. Group B is an open-label, multi-center study enrolling up to 16 patients with PH1 and PH2. Group B consists of three cohorts of patients with PH1 dosed at 1.5, 3 and 6 mg/kg, and an additional fourth cohort that consists of patients with PH2 dosed at flexible dosing levels. The fourth cohort runs in parallel to the other three cohorts. Dicerna has dosed 10 out of 16 patients in Group B—four PH1 patients in Cohort 1, four PH1 patients in Cohort 2, one PH1 patient in Cohort 3, and one PH2 patient in Cohort 4.
    - Dicerna completed the Group A portion of the study in NHVs. Group A is a placebo-controlled, single-blind, single center study that enrolled 25 NHVs. While the study remains blinded to treatment assignment, topline results from Group A evidence no serious adverse events (SAEs). 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 primary objective of PHYOX is to evaluate the safety and tolerability of single doses of DCR-PHXC in NHVs and patients with PH. The secondary objectives are to evaluate the pharmacodynamic effect of single doses of DCR-PHXC on urinary oxalate concentrations, as well as other biochemical markers, and to characterize the pharmacokinetics of single doses of DCR-PHXC in both groups.
    - Dicerna expects to report interim results from the PHYOX trial later in the third quarter and publicly present trial results in the fourth quarter of 2018. The Company intends to initiate a multi-dose Phase 2/3 study of DCR-PHXC in the first quarter of 2019, pending positive proof-of-concept data and regulatory feedback.
    - In May 2018, the U.S. Food and Drug Administration granted Orphan Drug Designation to DCR-PHXC for the treatment of PH.
    - In July 2018, the European Medicines Agency's Committee for Orphan Medicinal Products recommended designating DCR-PHXC as an orphan medicinal product for the treatment of PH in the European Union and the recommendation was adopted by the European Commission in August 2018.

- Chronic Hepatitis B Virus (HBV): Dicerna expects to file regulatory clearances for human clinical trials in New Zealand and Australia for DCR-HBVS, in development for the treatment of chronic HBV, during the fourth quarter of 2018. If the clearances are granted, the Company intends to begin clinical studies shortly thereafter.
- Undisclosed Rare Disease Involving the Liver: Dicerna is actively seeking a risk-sharing collaborator for this second program focused on a serious rare disease, before it files regulatory clearances to initiate a clinical trial. For competitive reasons, the Company has not yet publicly disclosed the target gene or disease.
- NASH Collaboration with Boehringer Ingelheim (BI): Dicerna continued to advance the program, initially focused on nonalcoholic steatohepatitis (NASH), in accordance with the work plan for this collaboration.

Conclusion of Litigation

- On April 18, 2018, Dicerna and Alnylam Pharmaceuticals 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 and neither party admitted wrongdoing as part of the Settlement Agreement. Pursuant to the terms of the Settlement Agreement, Dicerna paid to Alnylam an upfront payment of $2.0 million, agreed to a future payment of $13.0 million and issued 983,208 shares of common stock to Alnylam.

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 June 30, 2018, which was filed with the Securities and Exchange Commission (SEC) on August 8, 2018.

Financial Condition and Operating Results

- **Cash Position** – As of June 30, 2018, Dicerna had $82.3 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 June 30, 2018 and December 31, 2017, which reflects collateral securing the Company's operating lease obligation.
- **Revenue** – Dicerna recognized $1.5 million and $3.1 million of revenue associated with the BI Agreement, during the three and six months ended June 30, 2018, respectively. The revenue 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 were $10.3 million and $20.2 million for the three and six months ended June 30, 2018, respectively, as compared to $9.1 million and $17.8 million for the same periods in 2017. The increase was due to higher direct R&D expenses, partially offset by a reduction in platform-related expenses.

  The increase in the three months ended June 30, 2018 was primarily due to an increase in clinical study costs and an increase in R&D salaries due to an increase in R&D headcount compared to the same period in 2017. The increase in the six months ended June 30, 2018 was primarily due to an increase in toxicology studies and an increase in clinical studies.

  The decrease in platform-related expenses for the three and six months ended June 30, 2018 was primarily due to lab material and supply costs and contract research costs associated with applying the Company's GalXC platform against gene targets.

- **General and Administrative (G&A) Expenses** – G&A expenses were $4.8 million and $9.1 million for the three and six months ended June 30, 2018, respectively, as compared to $4.1 million and $8.2 million for the same periods in 2017. The increase is predominantly related to higher consulting and corporate legal expenses.
- **Litigation Expenses** – Litigation expenses, all related to the litigation with Alnylam, were $22.2 million and $25.4 million for the three and six months ended June 30, 2018, respectively, as compared to $2.2 million and $3.6 million for the same periods in 2017. The increase is predominantly due to the $21.0 million of settlement expenses related to the litigation settlement recorded in the second quarter of 2018.
- **Net Loss Attributable to Common Stockholders** – Net loss attributable to common stockholders was $35.6 million and $51.2 million for the three and six months ended June 30, 2018, respectively, as compared to $24.0 million and $38.2 million for the same periods in 2017. The increase is attributable to higher operating expenses, including the $21.0 million in litigation settlement expenses, partially offset by higher revenues, interest income and a decrease in dividends as a result of the conversion of the redeemable convertible preferred stock in December 2017.

For more detailed information and analysis, see Dicerna's Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, which was filed with the SEC on August 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 advancing DCR-PHXC through proof-of-concept trials and into late-stage clinical development and initiating proof-of-concept studies of DCR-HBVS in patients with HBV. 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 second 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 6588739 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 6588739. 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 the treatment of 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. The 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, expectations for discussions and possible opportunities with potential partners and collaborators, 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.
Condensed Consolidated Balance Sheet Information
(Unaudited, in thousands)

	June 30, 2018	December 31, 2017
Cash and cash equivalents	$ 42,426	$ 68,789
Held-to-maturity investments	$ 39,875	$ 44,889
Total assets	$ 89,715	$ 121,002
Total liabilities	$ 25,140	$ 19,916
Total stockholders' equity	$ 64,575	$ 101,086

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue from collaborative arrangements	$ 1,545	$ —	$ 3,090	$ —
Operating expenses:				
Research and development	10,339	9,068	20,232	17,811
General and administrative	4,760	4,066	9,095	8,188
Litigation expense	22,244	2,234	25,428	3,608
Total operating expenses	37,343	15,368	54,755	29,607
Loss from operations	(35,798)	(15,368)	(51,665)	(29,607)

Other income (expense):				
Interest income	330	143	619	181
Interest expense	(176)	—	(176)	—
Total other income, net	154	143	443	181
Net loss	(35,644)	(15,225)	(51,222)	(29,426)
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	$ (35,644)	$ (23,991)	$ (51,222)	$ (38,192)
Net loss per share attributable to common stockholders— basic and diluted	$ (0.68)	$ (1.15)	$ (0.98)	$ (1.84)
Weighted average common shares outstanding—basic and diluted	52,555,751	20,794,193	52,141,849	20,792,925

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Source: Dicerna Pharmaceuticals, Inc.

Investor:
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