Dicerna™ Reports First Quarter 2019 Financial and Operating Results and Provides Corporate Update

May 9, 2019

— Initiated Screening of Participants to Enroll in the PHYOX™2 Phase 2 Pivotal Trial of DCR-PHXC for the Treatment of Primary Hyperoxaluria (PH) and Announced Updated Data from Ongoing PHYOX1 Phase 1 of DCR-PHXC –

— Dosed First Healthy Volunteer in Phase 1 Clinical Trial of DCR-HBVS for Treatment of Chronic Hepatitis B Virus (HBV) Infection –

— Advanced Boehringer Ingelheim Collaboration with Exercise of Option for Second Hepatic Disease Target for Non-Alcoholic Steatohepatitis (NASH) and Other Chronic Liver Diseases —

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 9, 2019-- Dicerna™Pharmaceuticals, Inc. (Nasdaq: DRNA) (the “Company” or “Dicerna”), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operating results for the first quarter ended March 31, 2019.

“During the first quarter we were very pleased to report several important milestones for our lead GalXC™ program, DCR-PHXC, currently in development for primary hyperoxaluria, as well as other pipeline programs. In addition, we made strategic progress in augmenting our board of directors and management team with talented, experienced industry experts,” said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. “Specifically, we announced encouraging updated data demonstrating utility of DCR-PHXC from our PHYOX1 Phase 1 clinical trial at the German Society of Pediatric Nephrology 50th Annual Meeting in March. Based on these data, we began screening participants for our PHYOX2 Phase 2 pivotal trial for DCR-PHXC. We also advanced our Phase 1 DCR-HBVS program for the treatment of chronic hepatitis B virus infection by dosing our first healthy volunteer in the trial.”

“We are encouraged that Boehringer Ingelheim elected and is moving forward with a second GalXC compound against a hepatic target in the chronic liver disease space. Our recent strategic alliances with Alexion and Lilly are off to a strong start, as we have initiated nine early-stage discovery programs to generate GalXC molecules against both liver and neurological targets. Our ability to simultaneously drive discovery in these collaborative programs demonstrates the efficiency, robustness, and reproducibility of our GalXC discovery and optimization methods. We expect 2019 to be a year of execution for Dicerna, and we look forward to achieving key milestones in the second quarter, as we expect to dose the first patients in PHYOX2, PHYOX3 and our DCR-HBVS program and submit a clinical trial application for our second rare disease program.”

First Quarter 2019 Achievements

Clinical

- Initiated screening to enroll participants in the PHYOX2 Phase 2 multi-dose, double-blind, randomized, placebo-controlled pivotal trial of DCR-PHXC.
- Announced updated data from ongoing PHYOX1 Phase 1 clinical trial demonstrating utility of lead compound DCR-PHXC in treating primary hyperoxaluria Type 1 (PH1) and Type 2 (PH2). Data showed significant substantial post-dose reductions in 24-hour urinary oxalate levels in adult and adolescent study participants with PH1 and PH2.
    - Updated data for DCR-PHXC in 25 adult healthy volunteers (HVs) and 18 participants (15 adults and three adolescents [participants 12-17 years old]) with PH1 (n=15) and PH2 (n=3) were presented in a poster on March 28 at the German Society of Pediatric Nephrology 50th Annual Meeting in Cologne, Germany.
    - The results (as of March 14, 2019) show that a single dose of 3.0-mg/kg of DCR-PHXC brought urinary oxalate levels into the normal range (defined as 24-hour excretion <0.46 mmol) at one or more post-dose time points in four of five participants with PH1, including a mean maximal reduction of 24-hour urinary oxalate of 71% for the cohort. A single 1.5-mg/kg dose led to near-normalization (defined as 24-hour excretion <0.6 and ≥0.46 mmol) in three of five participants with PH1 dosed at this level, including a mean maximal reduction in urinary oxalate of 51% for the cohort. Among the three participants with PH1 dosed at 6.0-mg/kg, the mean maximal reduction in urinary oxalate was 76%. One participant in this cohort reached normalization and a second reached near-normalization, at one or more post-dose time points; one has not reached Day 57 and the other is still in follow-up and may not yet have reached maximal 24-hour urinary oxalate reduction.
    - Investigators reported that DCR-PHXC was generally well-tolerated in HVs and participants with PH. As of a data cut on March 14, 2019, four serious adverse events have occurred in three participants, though none were deemed related to the study drug. Nine participants (27%) dosed with DCR-PHXC experienced mild or moderate injection site reactions, all of which resolved without intervention within 96 hours.
- Achieved agreement on the primary endpoint for the PHYOX2 pivotal clinical trial, which is enrolling patients with PH1 and PH2, and alignment with the FDA regarding the path to full approval for the treatment of patients with PH1, as conveyed during a recent FDA Type A meeting.
- Dosed first HV in the multi-dose, double-blind, randomized, placebo-controlled Phase 1 clinical trial of DCR-HBVS,

studying the Company's investigational GalXC-based therapy for the treatment of chronic HBV infection in adults.
- Continued to advance internal development of the wholly-owned undisclosed program for a second rare disease involving the liver.

Collaborations

- Triggered $5 million payment from Boehringer Ingelheim International GmbH (BI) upon exercise of option for second hepatic disease target under research and license agreement.
    - The collaboration, established in October 2017, aims to discover and develop novel GalXC RNAi therapeutics for the treatment of chronic liver diseases, with an initial focus on NASH.
    - Dicerna is eligible to receive development and commercial milestone payments, and royalties on worldwide net sales.

Business Highlights

- Strengthened board of directors with addition of Marc Kozin, formerly vice-chair and head of healthcare, LEK Consulting, and Anna Protopapas, president and chief executive officer of Mersana Therapeutics, Inc.
    - J. Kevin Buchi, former chief executive officer of Cephalon, Inc. and TetraLogic Pharmaceuticals Corp., was appointed as board chair.
- Fortified leadership team to support Dicerna's continued growth with the strategic hires of Regina DeTore Paglia as senior vice president of human resources and Hardean Achneck, M.D., as vice president, head of medical development.

Upcoming Regulatory and Clinical Milestones

- Dose first patient in PHYOX2, a multi-dose, double-blind, randomized, placebo-controlled pivotal trial of DCR-PHXC for the treatment of all forms of PH expected in the second quarter of 2019.
- Dose first patient in PHYOX3, the long-term, multi-dose open-label, roll-over extension initially for our Phase 1 study for the treatment of PH expected in the second quarter of 2019.
- Dose first patient in the Phase 1 clinical trial of DCR-HBVS for the treatment of patients with chronic HBV expected in the second quarter of 2019.
    - The Company anticipates human proof-of-concept data from the first cohort to be available in the fourth quarter of 2019
- Submit a CTA in the second quarter of 2019 for the Company's undisclosed second rare disease program to initiate clinical trials.

Financial Condition and Operating Results for the First Quarter of 2019

- **Cash Position** –As of March 31, 2019, Dicerna had $371.2 million in cash, cash equivalents and held-to-maturity investments, which included $94.5 million in net proceeds received in the first quarter of 2019 from our collaborations, as compared to $302.6 million in cash, cash equivalents and held-to-maturity investments as of December 31, 2018. Additionally, the Company had $3.5 million and $0.7 million of restricted cash equivalents as of March 31, 2019 and December 31, 2018, respectively, which reflects collateral securing the Company's lease obligations.
- **Revenue** –Dicerna recognized $3.1 million of revenue associated with its collaboration agreements with Lilly, Alexion and BI during the first quarter ended March 31, 2019 compared with $1.5 million in the same period in 2018.
- **Research and Development (R&D) Expenses** – R&D expenses were $21.6 million in the first quarter ended March 31, 2019, as compared to $9.9 million for the same period in 2018. The increase, as compared to the same period in 2018, was primarily due to increased direct R&D expenses and an increase in employee-related expenses due to an increase in headcount necessary to support our growth.
- **General and Administrative (G&A) Expenses** – G&A expenses were $9.7 million for the first quarter ended March 31, 2019, as compared to $4.3 million for the same period in 2018. The increase is predominantly related to increases in employee-related expenses, including stock-based compensation expense, as well as an increase in general and business development consulting expense.
- **Litigation Expenses** – There were no litigation expenses in the first quarter ended March 31, 2019, as compared with $3.2 million in the first quarter of 2018. Litigation expense was solely related to trade secret litigation that was settled in the second quarter of 2018.
- **Net Loss** – Net loss was $26.2 million, or $0.38 per share, for the first quarter ended March 31, 2019, as compared to $15.6 million, or $0.30 per share, for the same period in 2018. The increase for the three months ended March 31, 2019 was driven by the increase in operating expenses.

Guidance

- Dicerna believes that its current cash, cash-equivalents and held-to-maturity investments will be sufficient to fund the execution of its current clinical and operating plan beyond 2020, which includes advancing DCR-PHXC through late-stage

clinical development and regulatory filing, completing proof-of-concept studies of DCR-HBVS in participants with HBV, and advancing the Company's undisclosed rare disease program through initial clinical studies. This estimate assumes no new funding from additional collaboration agreements or from external financing events and no significant unanticipated changes in costs and expenses.

- Dicerna expects its overall R&D expense to continue to increase for the foreseeable future, primarily as the Company completes clinical manufacturing activities, increases clinical and non-clinical development activities associated with its lead product candidates, and continues activities under the Lilly, Alexion and BI agreements.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's first quarter 2019 financial results and provide a general business update. The conference call can be accessed by dialing (855) 453-3834 or +1 (484) 756-4306 (international) and referencing conference ID 4749943 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 4749943. 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, 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 these genes 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. Dicerna has strategic collaborations with Eli Lilly and Company, Alexion Pharmaceuticals, Inc. and Boehringer Ingelheim International GmbH. 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 involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases 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. 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. 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. Examples of forward-looking statements include, among others, statements we make regarding: (i) the therapeutic and commercial potential of DCR-PHXC, DCR-HBVS and the GalXC™ platform; (ii) research and development plans and timelines related to DCR-PHXC, DCR-HBVS and GalXC; and (iii) the potential of Dicerna's technology and drug candidates in the Company's research and development pipeline. The process by which an early stage investigational therapy such as DCR-HBVS and an early stage platform such as GalXC could potentially lead to an approved product is long and subject to highly significant risks. Applicable risks and uncertainties include those relating to Dicerna's clinical research and other risks identified under the heading "Risk Factors" included in the Company's most recent Form 10-Q or 10-K filing and in other future filings with the Securities and Exchange Commission. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities; the likelihood of Dicerna's clinical programs being executed on timelines provided and reliance on the Company's contract research organizations and predictability of timely enrollment of subjects and patients to advance Dicerna's clinical trials; the potential for future data to alter initial and preliminary results of early stage clinical trials; the unpredictability of the duration and results of the regulatory review of Investigational New Drug Applications (NDAs) and CTAs that are necessary to continue to advance and progress the Company's clinical programs and the regulatory review of NDAs; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain and costs to obtain intellectual property rights; and possible safety or efficacy concerns that could emerge as new data are generated in R&D, general business, financial and accounting risks and litigation. 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™, GalXC™, and PHYOX™ ar trademarks of Dicerna Pharmaceuticals, Inc.

DICERNA PHARMACEUTICALS, INC.

SELECTED FINANCIAL INFORMATION (UNAUDITED)

Condensed Consolidated Balance Sheets	**March 31,**	**December 31,**

(In thousands)	2019	2019
Cash and cash equivalents	$ 160,141	$ 54,239
Held-to-maturity investments	211,063	248,387
Contract receivables	—	100,000
Other current assets	2,160	2,888
Right-of-use asset	3,047	—
Property and equipment, net	3,971	2,718
Restricted cash equivalents	3,544	744
Other noncurrent assets	63	65
Total Assets	$ 383,989	$ 409,041
Accounts payable	$ 4,958	$ 5,013
Accrued expenses and other current liabilities	8,196	9,649
Litigation settlement payable	—	10,500
Current portion of deferred revenue	59,784	68,893
Lease liability, current portion	1,750	—
Deferred revenue, net of current portion	128,295	114,293
Lease liability, noncurrent	1,378	—
Total stockholders' equity	179,628	200,693
Total Liabilities and Stockholders' Equity	$ 383,989	$ 409,041
Common stock outstanding	68,288,906	68,210,742

DICERNA PHARMACEUTICALS, INC.

SELECTED FINANCIAL INFORMATION (UNAUDITED)

Condensed Consolidated Statements of Operations

(In thousands, except per share data)	Three Months Ended March 31, 2019	Three Months Ended March 31, 2018
Revenue from collaborative arrangements	$ 3,107	$ 1,545
Operating expenses:		
Research and development	21,603	9,893
General and administrative	9,676	4,335
Litigation expense	—	3,184
Total operating expenses	31,279	17,412
Loss from operations	(28,172)	(15,867)
Interest income	2,018	288
Net loss	$ (26,154)	$ (15,579)
Net loss per share – basic and diluted	$ (0.38)	$ (0.30)
Weighted average common shares outstanding – basic and diluted	68,259	51,723

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Source: Dicerna™Pharmaceuticals, Inc.

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