



## Dicerna™ Reports Second Quarter 2019 Financial Results and Provides Corporate Update

August 8, 2019

- *Strengthened Management with Senior Hires Across Medical Affairs, Commercial, Regulatory, and Patient Advocacy and Patient Services* —
- *Initiated dosing in PHYOX™3 Roll-over Extension Study for the Treatment of Primary Hyperoxaluria (PH)* —
- *Received Breakthrough Therapy Designation for DCR-PHXC for Treatment of PH1* —
- *Announced Clinical Development Plans for DCR-A1AT for Treatment of Patients with Alpha-1 Antitrypsin Deficiency-Associated Liver Disease* —
- *Management to Host Conference Call Today at 3:30 p.m. ET* —

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 8, 2019-- [Dicerna™Pharmaceuticals, Inc.](#) (Nasdaq: DRNA) (the “Company” or “Dicerna”), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial results for the second quarter ended June 30, 2019 and provided a corporate update.

“Dicerna is dynamically growing as we move into pivotal development of our lead program in primary hyperoxaluria with an eye toward commercialization. Simultaneously, we’re increasing clinical development and discovery efforts for our own proprietary programs and those in conjunction with our corporate collaborators while expanding the reach of our GalXC RNAi technology,” said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. “I’m particularly pleased with the strength and depth of the new leaders who have joined Dicerna to drive these efforts. Notably, Dr. Bernd Hoppe, a renowned global expert in the treatment of primary hyperoxaluria, has joined us as vice president of global medical affairs, and we’re also joined by Rob Ciappenelli as chief commercial officer, Steven Kates as vice president of regulatory affairs, and Dave Caponera as head of patient advocacy and patient services. With these individuals and the rest of our team, we are confident that we will achieve our milestones and deliver innovative and effective therapies to patients in need.”

### Recent Clinical and Regulatory Highlights

- Initiated dosing in PHYOX™3, a long-term, multi-dose, open label, roll-over extension initially for our Phase 1 study for the treatment of PH.
- Received Breakthrough Therapy Designation for DCR-PHXC for the treatment of primary hyperoxaluria type 1 (PH1).
- Initiated PHYOX2, a multi-dose, double-blind, randomized, placebo-controlled pivotal trial of DCR-PHXC, which is being investigated for the treatment of all forms of PH.
- Presented additional data from PHYOX1 study in patients with PH1 and PH type 2 showing substantial reduction in urinary oxalate following single-dose administration of DCR-PHXC at the Oxalosis and Hyperoxaluria Foundation International Hyperoxaluria Workshop.
- Dosed first patient in the Phase 1 clinical trial of DCR-HBVS for the treatment of patients with chronic hepatitis B virus (HBV) infection.
- Submitted a clinical trial application to the Swedish Medical Products Agency for DCR-A1AT for the treatment of patients with alpha-1 antitrypsin deficiency-associated (A1AT) liver disease.

### Upcoming Clinical and Regulatory Milestones

- Dosing of first patient in the pivotal PHYOX2 trial.
- Human proof-of-concept data for DCR-HBVS in the first cohort of patients anticipated to be available in the fourth quarter of 2019.
- Initiation of a multi-center Phase 1/2 trial of DCR-A1AT is expected in the third quarter of 2019. The proposed parallel-group, placebo-controlled study will evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of DCR-A1AT in adult healthy volunteers (HVs) and patients with A1AT deficiency-associated liver disease.

### Financial Condition and Operating Results for the Second Quarter of 2019

- **Cash Position** —As of June 30, 2019, Dicerna had \$345.3 million in cash, cash equivalents, and held-to-maturity investments compared to \$302.6 million as of December 31, 2018. Additionally, the Company had \$3.5 million and \$0.7 million of restricted cash equivalents as of June 30, 2019 and December 31, 2018, respectively, reflecting collateral securing the Company’s lease obligations.
- **Revenue** —Dicerna recognized \$5.7 million of revenue associated with its collaboration agreements with Eli Lilly and Company (Lilly), Alexion Pharmaceuticals, Inc. (together with its affiliates, Alexion), and Boehringer Ingelheim International GmbH (BI) during the second quarter ended June 30, 2019 compared with \$1.5 million associated solely with BI in the same period in 2018.
- **Research and Development (R&D) Expenses** — R&D expenses were \$22.8 million in the second quarter ended June 30, 2019 compared to \$10.3 million for the same period in 2018. The increase was primarily due to increased direct R&D

costs, as well as employee-related expenses resulting from an increase in headcount necessary to support our growth.

- **General and Administrative (G&A) Expenses** – G&A expenses were \$8.8 million for the second quarter ended June 30, 2019 compared to \$4.8 million for the same period in 2018. The increase is predominantly due to employee-related expenses, as a result of increased stock-based compensation expense and headcount necessary to support our growth, as well as an increase in general and business development consulting expenses.
- **Net Loss** – Net loss was \$23.8 million, or \$0.35 per share, for the second quarter ended June 30, 2019 compared to \$35.6 million, or \$0.68 per share, for the same period in 2018. The decrease for the three months ended June 30, 2019 was primarily due to the absence of litigation expenses along with increased revenues in the second quarter of 2019, partially offset by increases in R&D and G&A 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 the proof-of-concept study of DCR-HBVS in participants with HBV, and advancing the Company's DCR-A1AT program through the initial clinical study. 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, advances preclinical toxicology studies, continues clinical activities associated with its lead product candidates, and increases 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 second 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 6279126 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](http://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 6279126. The webcast will also be archived on Dicerna's website.

## About Dicerna™Pharmaceuticals, Inc.

Dicerna™Pharmaceuticals, Inc. ("we," "us," "our," "the Company," or "Dicerna") is a biopharmaceutical company using ribonucleic acid (RNA) interference (RNAi) to develop medicines that silence genes that cause disease. The Company's proprietary GalXC™ technology is being applied to develop potent, selective, and safe RNAi therapies to treat diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. Dicerna aims to treat disease by addressing the underlying causes of illness with capabilities that extend beyond the liver to address a broad range of diseases, focusing on target genes where connections between gene and disease 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 (Lilly), Alexion Pharmaceuticals, Inc. (Alexion), and Boehringer Ingelheim International GmbH (BI). For more information, please visit [www.dicerna.com](http://www.dicerna.com).

## About GalXC™

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, DCR-A1AT, and the GalXC™ platform; (ii) research and development plans and timelines related to DCR-PHXC, DCR-HBVS, DCR-A1AT, and GalXC; (iii) the potential of Dicerna's technology and drug candidates in the Company's research and development pipeline, and (iv) Dicerna's financial position and cash runway. The process by which an early stage investigational therapy such as DCR-PHXC, DCR-HBVS, DCR-A1AT, 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 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 future agreement on clinical endpoints for all forms of PH, the unpredictability of the duration and results of the regulatory review of Investigational New Drug Applications (NDAs) and Clinical Trial Applications 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; possible safety or efficacy concerns that could emerge as new data are generated in R&D; and 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™ are trademarks of Dicerna Pharmaceuticals, Inc.

**DICERNA PHARMACEUTICALS, INC.  
SELECTED FINANCIAL INFORMATION (UNAUDITED)**

<b>Condensed Consolidated Balance Sheets</b>	<b>June 30,</b>	<b>December 31,</b>
<b>(In thousands, except share data)</b>	<b>2019</b>	<b>2018</b>
Cash and cash equivalents	\$ 100,321	\$ 54,239
Held-to-maturity investments	244,969	248,387
Contract receivables	—	100,000
Prepaid expenses and other current assets	4,958	2,888
Property and equipment, net	4,915	2,718
Right-of-use asset	2,627	—
Restricted cash equivalents	3,544	744
Other noncurrent assets	62	65
<b>Total Assets</b>	<b>\$ 361,396</b>	<b>\$ 409,041</b>
Accounts payable	\$ 5,636	\$ 5,013
Accrued expenses and other current liabilities	10,515	9,649
Lease liability, current	1,648	—
Litigation settlement payable	—	10,500
Deferred revenue, current	78,073	68,893
Lease liability, noncurrent	1,035	—
Deferred revenue, noncurrent	104,324	114,293
Total stockholders' equity	160,165	200,693
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 361,396</b>	<b>\$ 409,041</b>
Common stock outstanding	68,360,051	68,210,742

**Condensed Consolidated Statements of Operations**

**Three Months Ended Three Months Ended**

<b>(In thousands, except per share data)</b>	<b>June 30, 2019</b>	<b>June 30, 2018</b>
Revenue from collaborative arrangements	\$ 5,682	\$ 1,545
Operating expenses:		
Research and development	22,832	10,339
General and administrative	8,831	4,760
Litigation expense	—	22,244
Total operating expenses	31,663	37,343
Loss from operations	(25,981	) (35,798
Other income (expense):		
Interest income	2,136	330
Interest expense	—	(176
Total other income, net	2,136	154
Net loss	\$ (23,845	) \$ (35,644
Net loss per share – basic and diluted	\$ (0.35	) \$ (0.68
Weighted average common shares outstanding – basic and diluted	68,323,850	52,555,751

<b>Condensed Consolidated Statements of Operations</b>	<b>Six Months Ended</b>	<b>Six Months Ended</b>
<b>(In thousands, except per share data)</b>	<b>June 30, 2019</b>	<b>June 30, 2018</b>
Revenue from collaborative arrangements	\$ 8,789	\$ 3,090
Operating expenses:		
Research and development	44,435	20,232
General and administrative	18,507	9,095
Litigation expense	—	25,428
Total operating expenses	62,942	54,755
Loss from operations	(54,153	) (51,665
Other income (expense):		
Interest income	4,154	619
Interest expense	—	(176
Total other income, net	4,154	443
Net loss	\$ (49,999	) \$ (51,222

Net loss per share – basic and diluted	\$ (0.73	) \$ (0.98	)
Weighted average common shares outstanding – basic and diluted	68,292,212	52,141,849	

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