Dicerna™ Announces Third Quarter 2019 Financial Results and Reports Development Programs Progress

November 7, 2019

—Initiated Dosing of Patients With Primary Hyperoxaluria Types 1 and 2 in PHYOX™2 Pivotal Clinical Trial —

—Announced Collaboration Agreement With Roche for Development of DCR-HBVS Including Option to Co-Fund Pivotal Development and Co-Promote in the U.S. —

—$312.7 Million in Cash, Cash Equivalents, and Marketable Securities at September 30, 2019; Dicerna to Receive $200.0 Million Upon Close of HBV Agreement with Roche —

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 7, 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 third quarter ended September 30, 2019 and provided a corporate update.

"Dicerna has recently achieved two significant strategic objectives: dosing of patients in our PHYOX2 pivotal clinical trial and entering a collaboration agreement for the development of treatments for chronic hepatitis B virus infection with Roche, a global leader in the field," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "We continue to maintain full control of our lead rare disease programs for the treatment of all types of primary hyperoxaluria and for alpha-1 antitrypsin deficiency-associated liver disease. In parallel, our collaboration with Roche for HBV infection will generate near-term resources that will be used to grow our business, including our evolving commercial efforts. The Roche collaboration also offers us the option, after Phase 2 clinical proof-of-efficacy combination studies, to co-fund pivotal development and co-commercialize DCR-HBVS. This portfolio reflects our strategy to minimize our exposure to clinical risk while maximizing our commercial rights. Our three existing collaborations with Lilly, Alexion, and Boehringer Ingelheim have provided significant resources to advance this strategy while enabling the development of programs for larger disease populations that we would not have pursued on our own.

"Together, this portfolio of programs and collaborations is a testament to the power and versatility of our GalXC RNAi platform," continued Dr. Fambrough. "Going forward, we believe there remains significant potential for additional programs and collaborative work with our liver-targeted GalXC technology, while we also continue to expand the reach of our GalXC technology to additional tissues."

Recent Corporate Achievements

- Announced a research collaboration and licensing agreement with Roche to develop novel therapies for the treatment of chronic hepatitis B virus (HBV) infection using Dicerna's proprietary GalXC™ RNAi platform technology. The collaboration will focus on worldwide development and commercialization of DCR-HBVS, Dicerna's investigational therapy in Phase 1 clinical development. The agreement also includes the discovery and development of therapies targeting human genes associated with HBV infection, or additional targets within the HBV genome, using the technology platforms of both companies. Under the collaboration, Dicerna will receive $200 million up front plus up to $1.47 billion in potential milestone payments related to DCR-HBVS.
- Initiated dosing in PHYOX™2, a multi-dose, double-blind, randomized, placebo-controlled pivotal trial of DCR-PHXC that is expected to enroll approximately 36 patients with primary hyperoxaluria (PH) type 1 and type 2 (PH1 and PH2).
- Received feedback from the U.S. Food and Drug Administration indicating alignment on a path to full approval of DCR-PHXC for the treatment of PH2 based on achievement of substantial reduction of high baseline urinary oxalate in patients with PH2 in the PHYOX2 pivotal trial. With this feedback, we believe we have a path to full approval in both PH1 and PH2 based on PHYOX2 results.

Upcoming Milestones

- Enrollment of first healthy volunteers in Phase 1/2 trial of DCR-A1AT for treatment of patients with alpha-1 antitrypsin deficiency-associated liver disease expected in the fourth quarter of 2019.
- Multi-dose data from PHYOX3, a long-term, multi-dose, open-label, roll-over extension from our Phase 1 study for the treatment of PH expected in the first half of 2020.
- Completion of enrollment of our PHYOX2 pivotal clinical trial by the end of the first half of 2020.
- Proof-of-concept data from all planned cohorts of our DCR-HBVS Phase 1 clinical trial in mid-2020.
- Development of additional programs and collaborative work with our liver-targeted GalXC technology and expansion of the reach of our GalXC technology to additional tissues.

Financial Condition and Operating Results for the Third Quarter of 2019

- **Cash Position** – As of September 30, 2019, Dicerna had $312.7 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 September 30, 2019 and December 31, 2018, respectively, reflecting collateral securing the Company's lease obligations.

- **Revenue** – Dicerna recognized $8.0 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 quarter ended September 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 $30.1 million in the quarter ended September 30, 2019, compared to $11.7 million for the same period in 2018. The increase was primarily due to increased manufacturing costs, clinical study costs, and employee-related expenses due to an increase in headcount necessary to support our growth.
- **General and Administrative (G&A) Expenses** – G&A expenses were $10.6 million for the quarter ended September 30, 2019, compared to $5.4 million for the same period in 2018. The increase is primarily 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 $30.8 million, or $0.45 per share, for the quarter ended September 30, 2019, compared to $19.0 million, or $0.35 per share, for the same period in 2018.

Guidance

Dicerna believes that its cash, cash-equivalents, and held-to-maturity investments along with the $200.0 million upfront payment expected upon closing of the recently announced agreement with Roche, will be sufficient to fund the execution of its current clinical and operating plan beyond 2021, which includes advancing DCR-PHXC through pivotal development, regulatory filing, and potential commercial launch; completing proof-of-concept studies of DCR-HBVS in participants with HBV infection; and advancing the Company's DCR-A1AT program through the initial Phase 1/2 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 expenses to continue to increase for the foreseeable future, primarily as the Company completes clinical manufacturing activities, continues clinical activities associated with its lead product candidates, advances preclinical toxicology studies, increases activities under the Lilly, Alexion, and BI agreements, and prepares for commercialization of DCR-PHXC.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's third 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 1475058 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 30 days. To access the replay, please dial (855) 859-2056 or (404) 537-3406 and refer to conference ID 1475058. The webcast will also be archived on Dicerna's website.

About Dicerna™Pharmaceuticals, Inc.

Dicerna™Pharmaceuticals, Inc., 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 for treatment of rare diseases, chronic liver diseases, cardiovascular diseases, neurodegenerative diseases, pain, and viral infectious disease. 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 therapies either on its own or in collaboration with pharmaceutical partners. Dicerna has strategic collaborations with Roche, Eli Lilly and Company, Alexion Pharmaceuticals, Inc., and Boehringer Ingelheim International GmbH. For more information, please visit www.dicerna.com.

About Dicerna's GalXC™ RNAi Technology Platform

Dicerna's proprietary RNA interference (RNAi) technology platform, called GalXC™, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver and other body systems. Liver-targeted GalXC-based compounds enable subcutaneous delivery of RNAi therapies that are designed to specifically bind to receptors on liver cells, leading to internalization and access to the RNAi machinery within the cells. The GalXC approach seeks to optimize the activity of the RNAi pathway so that it operates in the most specific and potent fashion. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, including both liver and non-liver indications.

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, including continued alignment on the regulatory approval of DCR-PHXC; (iii) the potential for Dicerna to add programs and expand collaborative work with our liver-targeted GalXC technology and to extend the reach of our GalXC technology to additional tissues; (iv) the potential of Dicerna's technology and drug candidates in the Company's research and development pipeline; and (v) Dicerna's financial position, expectations about current or future collaboration funding, expenses, 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 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 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS (Unaudited)

(in thousands, except share data)

	SEPTEMBER 30, 2019	DECEMBER 31, 2018
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 47,226	$ 54,239
Held-to-maturity investments	265,484	248,387
Contract receivables	3,000	100,000
Prepaid expenses and other current assets	4,688	2,888
Total current assets	320,398	405,514
NONCURRENT ASSETS:		
Property and equipment, net	5,508	2,718
Right-of-use assets	2,224	—
Restricted cash equivalents	3,544	744
Other noncurrent assets	4,576	65
Total noncurrent assets	15,852	3,527
TOTAL ASSETS	$ 336,250	$ 409,041
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 3,407	$ 5,013
Accrued expenses and other current liabilities	18,585	9,649

Lease liability, current	1,710	—
Litigation settlement payable	—	10,500
Deferred revenue, current	95,659	68,893
Total current liabilities	119,361	94,055
NONCURRENT LIABILITIES:		
Lease liability, noncurrent	545	—
Deferred revenue, noncurrent	81,977	114,293
Total noncurrent liabilities	82,522	114,293
TOTAL LIABILITIES	201,883	208,348
COMMITMENTS AND CONTINGENCIES (NOTE 10)		
STOCKHOLDERS' EQUITY:		
Preferred stock, $0.0001 par value – 5,000,000 shares authorized; no shares issued or outstanding at September 30, 2019 or December 31, 2018	—	—
Common stock, $0.0001 par value – 150,000,000 shares authorized; 68,360,051 and 68,210,742 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	7	7
Additional paid-in capital	620,009	605,495
Accumulated other comprehensive loss	(3)	—
Accumulated deficit	(485,646)	(404,809)
Total stockholders' equity	134,367	200,693
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 336,250	$ 409,041

DICERNA PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (Unaudited)

(in thousands, except share and per share data)

	THREE MONTHS ENDED SEPTEMBER 30,		**NINE MONTHS ENDED SEPTEMBER 30,**	
	2019	**2018**	**2019**	**2018**
Revenue from collaborative arrangements	$ 8,035	$ 1,545	$ 16,824	$ 4,635
Operating expenses:				

Research and development	30,086	11,695	74,521	31,927
General and administrative	10,619	5,354	29,126	14,449
Litigation expense	—	3,694	—	29,122
Total operating expenses	40,705	20,743	103,647	75,498
Loss from operations	(32,670)	(19,198)	(86,823)	(70,863)
Other income (expense):				
Interest income	1,880	401	6,034	1,020
Interest expense	—	(223)	—	(399)
Total other income, net	1,880	178	6,034	621
Net loss	$ (30,790)	$ (19,020)	$ (80,789)	$ (70,242)
Foreign currency translation adjustment	(3)	—	(3)	—
Comprehensive loss	$ (30,793)	$ (19,020)	$ (80,792)	$ (70,242)
Net loss per share – basic and diluted	$ (0.45)	$ (0.35)	$ (1.18)	$ (1.32)
Weighted average common shares outstanding – basic and diluted	68,360,051	54,799,644	68,315,074	53,037,516

View source version on businesswire.com: https://www.businesswire.com/news/home/20191107006026/en/

Source: Dicerna™Pharmaceuticals, Inc.

Investors:
Stern Investor Relations, Inc.
Lauren Stival, 212-362-1200
lauren.stival@sternir.com

Media:
SmithSolve
Alex Van Rees, 973-442-1555 ext. 111
alex.vanrees@smithsolve.com