Dicerna Announces Second Quarter 2020 Financial Results and Provides a Business Update

August 6, 2020

– Company Targeting Enrollment Completion for Pivotal PHYOX™2 Trial of Nedosiran in Fourth Quarter 2020 –

– Company Reported $669.2 Million in Cash, Cash Equivalents and Marketable Securities as of June 30, 2020 –

– Management to Host Virtual R&D Day Event Today at 10:00 a.m. ET –

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 6, 2020-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) (the "Company" or "Dicerna"), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported its financial results for the second quarter ended June 30, 2020.

"Our organization's steady and consistent execution across all fronts, despite the impacts of COVID-19 on all facets of daily life, continues to advance our goals of expanding the reach of RNAi into numerous therapeutic categories and becoming a commercial-stage organization. The efforts of our employees, clinical sites, manufacturing and collaborative partners demonstrate an important shared dedication to bringing new RNAi-based therapies to patients in need," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "In addition to our financial results, today we also updated our guidance for completion of enrollment of our pivotal PHYOX2 trial of nedosiran in patients with primary hyperoxaluria types 1 or 2, to the fourth quarter of 2020. We look forward to discussing our clinical progress in detail at our R&D Day event today, which will include interim positive data from our ongoing PHYOX3 multidose trial of nedosiran, Phase 1 proof-of-concept data from our ongoing trial of RG6346 for the treatment of chronic hepatitis B virus infection, and our first public presentation of preclinical data demonstrating delivery of our proprietary technology to multiple tissues outside the liver – all of which reinforce Dicerna's position as a best-in-class company in RNAi therapeutics."

Clinical and Regulatory Updates

Since its first COVID-19-related update in March, the Company has continued to monitor developments related to the pandemic and potential impacts to its business and clinical development programs, including the timing of development milestones. Dicerna today provided an update on the progress of each of its core development programs and development timeline expectations.

- **RG6346 Phase 1 Proof-of-Concept Trial for Chronic Hepatitis B Virus Infection.** RG6346 is an investigational candidate for the treatment of chronic hepatitis B virus (HBV) infection being developed in collaboration with Roche. Enrollment of the Phase 1 clinical study of RG6346 was completed in June 2020. In a separate news release today, Dicerna announced positive data from the ongoing trial in which patients from its multidose Group C reached a mean reduction in hepatitis B surface antigen (HBsAg) of 1.39, 1.80 and 1.84 log10 IU/mL at Day 112 end-of-treatment for the 1.5, 3.0 and 6.0 mg/kg cohorts, respectively. Notably, HBsAg suppression has been stably maintained beyond Day 112, with the 1.5 mg/kg cohort showing stable mean HBsAg reduction through Day 336, confirming preclinical predictions of a long duration of effect for Dicerna's X-sparing HBV targeting strategy. No serious adverse events (SAEs) were observed with RG6346 treatment in any group. The most commonly reported adverse events were mild or moderate injection-site events. No dose-limiting toxicities were observed, and there were no safety-related discontinuations. Under the collaboration agreement, Roche will be responsible for initiating Phase 2 development of RG6346.

- **Positive Interim Data from PHYOX3 Multidose, Open-Label Extension Trial of Nedosiran.** In April 2020, in response to COVID-19 impacts at clinical trial sites, the Company implemented a protocol amendment with local Institutional Review Boards and transitioned certain site visits to a combination of at-home nurse visits with investigator telehealth assessments for dose administration and safety follow-up.

  Updated interim data from this ongoing trial, which were released in a separate news release this morning, demonstrated that of the 11 participants included in the analysis (eight PH1 and three PH2) who had reached Day 120 receiving once-monthly nedosiran, nine participants (82%) had achieved normal or near-normal urinary oxalate (Uox) levels, defined as below 0.46 mmol/1.73m^2 body surface area adjusted (BSA)/24 hr (laboratory assay upper limit of normal [ULN]) and from 0.46 to 0.6 mmol/1.73m^2 BSA/24 hr (1.3xULN, defined per protocol as near-normal), respectively. To date, five of the 17 participants enrolled in PHYOX3 had achieved and maintained normal Uox concentrations on at least three consecutive visits, making them eligible for gradual reduction in fluid intake. In this interim analysis, nedosiran appeared generally well tolerated, with three injection-site reactions in the 16 enrolled patients and no drug-related severe adverse events. The overall adverse event profile was comparable to that observed in the PHYOX1 Phase 1 clinical trial.

- **Nedosiran PHYOX2 Pivotal Trial.** In April and May 2020, Dicerna transitioned certain site visits in the PHYOX2 trial to a combination of at-home nurse visits and investigator telehealth assessments for drug administration and safety follow-up to align with trial sites' operational changes resulting from the COVID-19 pandemic. Enrollment in the PHYOX2 trial continues at a limited number of sites globally. While subject to change pending potential future site impacts related to the pandemic, the Company currently expects to complete enrollment in the PHYOX2 trial in the fourth quarter of 2020.

- **Rare Pediatric Disease Designation for Nedosiran for Treatment of Primary Hyperoxaluria.** In June 2020, the U.S. Food and Drug Administration (FDA) granted rare pediatric disease designation for nedosiran. Under the FDA's rare pediatric disease designation program, the FDA may grant a priority review voucher to a sponsor who receives a product approval for a “rare pediatric disease.” Subject to FDA approval of nedosiran for the treatment of PH, Dicerna would be eligible to receive a voucher that may be redeemed to receive priority review for a subsequent marketing application for a different product candidate or which could be sold or transferred.

- **Preclinical Data From Dicerna’s Technology in New Tissues.** In a separate news release today, Dicerna announced positive preclinical data demonstrating expansion of its technology and discovery efforts beyond its hepatocyte-focused GalXC RNAi technology to central nervous system (CNS), skeletal muscle and adipose tissues. The data demonstrated consistent and durable CNS-wide target mRNA knockdown using novel constructs regardless of route of administration (intrathecal [IT] or intracisterna magna [ICM]) and reduction in target mRNA (messenger RNA) in skeletal muscle and adipose tissue using optimized chemistries, resulting in equivalent and potentially highly durable target knockdown regardless of dosing regimens.

- **DCR-A1AT Phase 1/2 Trial and ALN-AAT02 Program Update.** In late April 2020, the Scientific Review Committee for the DCR-A1AT Phase 1/2 trial confirmed that the study could continue, and the Company began enrolling the following dosing cohort in May, which has since been completed. The Company is currently targeting program selection and potential initiation of patient dosing in the first quarter of 2021.

Corporate and Collaboration Program Updates

- **Shreeram Aradhye, M.D. Appointed Executive Vice President and Chief Medical Officer.** Dicerna announced yesterday the appointment of Dr. Aradhye as executive vice president and chief medical officer, effective Sept. 8, 2020. Dr. Aradhye brings over 20 years of pharmaceutical industry experience in clinical development and medical affairs, most recently as chief development officer of Axcella Health. Prior to Axcella, Dr. Aradhye served as global head of medical affairs and chief medical officer – pharmaceuticals for Novartis Pharmaceuticals.

- **Roche Collaboration Agreement.** In April 2020, Roche formally nominated the first selected target and thus initiated the research and development portion of its agreement with the Company. In October 2019, Dicerna and Roche entered into an agreement related to the development and commercialization of RG6346 and the discovery, development and commercialization of oligonucleotide therapeutics targeting multiple gene targets implicated in chronic hepatitis B virus (HBV) infection.

Upcoming Milestones

- **Nedosiran:** Expected completion of enrollment in PHYOX2 pivotal clinical trial in the fourth quarter of 2020 with last patient anticipated to complete the study in the first half of 2021 and subsequent New Drug Application submission in the third quarter of 2021

- **Collaborative Program:** Investigational New Drug or Clinical Trial Authorization filing for LY3561774 by Lilly in late 2020

- **DCR-A1AT/ ALN-AAT02:** Program selection and potential initiation of patient dosing in the first quarter of 2021

Supply Chain Update

The current supply of Dicerna’s investigational medicines continues to be sufficient to support ongoing clinical trials. Based on current evaluations, Dicerna’s supply chain continues to appear intact at this time to meet expected 2020 clinical, nonclinical, and chemistry, manufacturing and control supply demands across all programs. The Company has undertaken efforts to mitigate potential future impacts to the supply chain by increasing its stock of critical starting materials required to meet its needs and its collaborative partners’ needs through mid-2021 and by identifying and engaging alternative suppliers. The Company continues to be alert to the potential for disruptions that could arise from COVID-19 and remains in close contact with suppliers.

Financial Results for Second Quarter of 2020

- **Cash Position** – As of June 30, 2020, Dicerna had $669.2 million in cash, cash equivalents and held-to-maturity investments, compared to $348.9 million as of Dec. 31, 2019. Additionally, the Company had $5.6 million and $3.9 million of restricted cash equivalents as of June 30, 2020 and Dec. 31, 2019, respectively, reflecting collateral securing the Company’s lease obligations.

- **Revenue** – Dicerna recognized $40.4 million of revenue associated with its collaboration partners during the quarter ended June 30, 2020, compared to $5.7 million for the same period in 2019.

- **Research and Development (R&D) Expenses** – R&D expenses were $53.4 million for the quarter ended June 30, 2020, compared to $22.8 million for the same period in 2019. The increase was primarily due to direct research and development

expenses as a result of manufacturing and 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 $20.6 million for the quarter ended June 30, 2020, compared to $8.8 million for the same period in 2019. The increase was primarily due to employee-related expenses as a result of increased headcount necessary to support our growth.

- **Net Loss** – Net loss was $31.8 million, or $0.43 per share, for the quarter ended June 30, 2020, compared to $23.8 million, or $0.35 per share, for the same period in 2019.

Guidance

Dicerna believes that its cash, cash equivalents and held-to-maturity investments will be sufficient to fund the execution of its current clinical and operating plan into 2023, which includes our expectations to advance nedosiran through pivotal development, regulatory filing and potential commercial launch; completing the proof-of-concept study of RG6346 in participants with HBV infection; conducting nonclinical studies of ALN-AAT02 and advancing either ALN-AAT02 or DCR-A1AT through Phase 1/2; and initiating and conducting research and development programs with our collaborative partners. 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 significantly for the foreseeable future, primarily as the Company continues clinical manufacturing activities, advances preclinical toxicology studies, continues clinical activities associated with its lead product candidates, prepares for commercialization of nedosiran and initiates or increases activities under the agreements with Novo Nordisk A/S, Roche, Eli Lilly, Alexion Pharmaceuticals, Inc., Boehringer Ingelheim International GmbH and Alnylam Pharmaceuticals, Inc.

Virtual R&D Day Event Webcast Details

The Company will host a Virtual R&D Day Event to discuss the results from its Phase 1 proof-of-concept trial of RG6346, its PHYOX3 multidose trial of nedosiran and preclinical data related to its technology in extrahepatic tissues. The webcast presentation will begin at 10:00 a.m. ET, and may be accessed by visiting the “Investors & Media” section of the Dicerna website, www.dicerna.com. A conference line can be accessed by dialing (800) 708-4539 or +1 (847) 619-6396 and referencing conference ID 49860522. A replay of the webcast will be archived on Dicerna’s website following the conclusion of the live event.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) is a biopharmaceutical company focused on discovering, developing and commercializing medicines that are designed to leverage ribonucleic acid interference (RNAi) to selectively silence genes that cause or contribute to disease. Using our proprietary RNAi technology platform called GalXC™, Dicerna is committed to developing RNAi-based therapies with the potential to treat both rare and more prevalent diseases. By silencing disease-causing genes, Dicerna’s GalXC platform has the potential to address conditions that are difficult to treat with other modalities. Initially focused on hepatocytes, Dicerna has continued to innovate and is exploring new applications of its RNAi technology beyond the liver, targeting additional tissues and enabling new therapeutic applications. In addition to our own pipeline of core discovery and clinical candidates, Dicerna has established collaborative relationships with some of the world’s leading pharmaceutical companies, including Novo Nordisk A/S, Roche, Eli Lilly and Company, Alexion Pharmaceuticals, Inc., Boehringer Ingelheim International GmbH and Alnylam Pharmaceuticals, Inc. Between Dicerna and our collaborative partners, we currently have more than 20 active discovery, preclinical or clinical programs focused on rare, cardiometabolic, viral, chronic liver and complement-mediated diseases, as well as neurodegeneration and pain. At Dicerna, our mission is to interfere – to silence genes, to fight disease, to restore health. For more information, please visit www.dicerna.com.

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) Phase 1 proof-of-concept data for RG6346, an investigational GalXC™ RNAi treatment candidate for chronic hepatitis B virus (HBV) infection in development with Roche; (ii) multidose data from the PHYOX™3 trial of nedosiran, an investigational GalXC RNAi treatment candidate for primary hyperoxaluria (PH), (iii) preclinical data on Dicerna’s technology in extrahepatic tissues; (iv) the therapeutic and commercial potential of nedosiran; (v) guidance concerning future financial results, sufficient cash for future operations and corporate developments and (vi) clinical development timelines and review related to nedosiran and continued alignment on the regulatory pathway to approval. The process by which investigational therapies, such as nedosiran, 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 filings on Forms 10-K and 10-Q 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 by us and our collaborative partners; 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 reliance of Dicerna on contract manufacturers to supply its products for research and development and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the impact of the ongoing COVID-19 pandemic on our business operations, including the conduct of our research and development activities; the regulatory review and unpredictability of the duration and results of the regulatory review of Investigational New Drug applications (INDs) and Clinical Trial Applications (CTAs) that are necessary to continue to advance and progress the Company’s clinical programs; the timing, plans and reviews by regulatory authorities of marketing applications such as New Drug Applications (NDAs) and comparable foreign applications for one or more of Dicerna’s product candidates; the ability to secure, maintain and realize the intended benefits of collaborations with partners; 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.
SELECTED FINANCIAL INFORMATION (UNAUDITED)

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended June 30, 2020**	**Three Months Ended June 30, 2019**
Revenue	$ 40,448	$ 5,682
Operating expenses:		
Research and development	53,376	22,832
General and administrative	20,565	8,831
Total operating expenses	73,941	31,663
Loss from operations	(33,493)	(25,981)
Other income (expense):		
Interest income	1,729	2,136
Interest expense	(6)	—
Other expense	(50)	—
Total other income, net	1,673	2,136
Net loss	$ (31,820)	$ (23,845)
Net loss per share – basic and diluted	$ (0.43)	$ (0.35)
Weighted-average common shares outstanding – basic and diluted	74,001	68,324

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Six Months Ended June 30, 2020**	**Six Months Ended June 30, 2019**
Revenue	$ 74,476	$ 8,789
Operating expenses:		
Research and development	96,547	44,435
General and administrative	36,588	18,507
Total operating expenses	133,135	62,942
Loss from operations	(58,659)	(54,153)
Other income (expense):		
Interest income	4,342	4,154
Interest expense	(10)	—
Other income	15	—
Total other income, net	4,347	4,154
Net loss	$ (54,312)	$ (49,999)
Net loss per share – basic and diluted	$ (0.74)	$ (0.73)
Weighted-average common shares outstanding – basic and diluted	73,460	68,292

CONDENSED CONSOLIDATED BALANCE SHEETS (In thousands)	**June 30, 2020**	**December 31, 2019**
Cash and cash equivalents	$197,801	$ 152,816
Held-to-maturity investments	471,408	196,065
Contract receivables	12	200,354
Prepaid expenses and other current assets	12,541	6,934
Property and equipment, net	9,135	7,076
Right-of-use operating assets, net	29,488	30,102
Restricted cash equivalents	5,563	3,894
Other noncurrent assets	6,462	168
Total Assets	$732,410	$ 597,409
Accounts payable	$ 7,764	$ 6,077
Accrued expenses and other current liabilities	27,009	20,042
Lease liability, current	2,810	3,358
Deferred revenue, current	221,343	212,258
Lease liability, noncurrent	19,819	20,141
Deferred revenue, noncurrent	290,889	182,730
Other noncurrent liabilities	608	608
Total stockholders' equity	162,168	152,195
Total Liabilities and Stockholders' Equity	$732,410	$ 597,409

Common stock outstanding	74,321	71,573

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