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

May 7, 2020

— Company Continues to Advance Ongoing Nedosiran, DCR-A1AT and RG6346 Clinical Trials —

— Company Well Capitalized With $706.9 Million in Cash, Cash Equivalents and Marketable Securities as of March 31, 2020 –

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

LEXINGTON, Mass.--(BUSINESS WIRE)--May 7, 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 first quarter ended March 31, 2020.

"COVID-19 has undeniably impacted nearly every facet of life, including drug development. Despite the significant challenges the pandemic has presented for employees, participants in ongoing clinical studies, families and caregivers, and patient communities, we continued to make meaningful progress across our business in the first quarter thanks in large part to the dedication and adaptability of our employees," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "While COVID-19 has resulted in slower enrollment in our clinical trials, we have nonetheless been able to execute on every front within our immediate control and have been putting in place measures that we believe should help to mitigate the potential for any protracted effects from trial adjustments to allow us to rapidly move forward on a clinical level as restrictions are lifted and it is safe to do so.

"Among our recent achievements, we met our objective to deliver an overview of the positive early observations from our PHYOX™3 long-term, multidose trial of nedosiran, our lead product candidate in development for treatment of primary hyperoxaluria, or PH, types 1, 2 and 3," Dr. Fambrough continued. "We also entered into two agreements with Alnylam in early April – the first of which enhances our confidence to bring nedosiran to market upon approval and will enable us to earn meaningful royalties on product sales of Alnylam's PH type 1 product candidate post-approval; and the second of which provides us with another treatment candidate to evaluate for alpha-1 antitrypsin deficiency-associated liver disease, enhancing our opportunity to advance a new therapy that we believe has the greatest potential to benefit patients. We continue our efforts to steadily screen patients for enrollment in our PHYOX2 pivotal trial of nedosiran, and RG6346, our Phase 1 candidate for HBV that we are developing in collaboration with Roche, continues to progress toward enrollment completion. We are looking forward to presenting data from across our core development pipeline and platform at our upcoming R&D Day in August.

"While the COVID-19 situation and its future impacts remain uncertain on a macro level, at a corporate level, this global health crisis is bringing into focus the strengths of Dicerna's business model and the critical importance of innovative biotechnology to protecting and addressing public health. Our balance sheet is strong, and we have the capital on hand to appropriately resource each of the key functions necessary to execute on our goals, positioning us well as we continue our evolution toward becoming a fully integrated, commercial-stage biopharmaceutical company," Dr. Fambrough concluded.

Recent Events

- **Douglas Pagán to Become Chief Financial Officer.** In a separate news release issued today, Dicerna announced that Douglas Pagán will succeed Jack Green who is retiring as Dicerna's chief financial officer. Mr. Pagán will begin his new role with the Company on May 26, 2020.
- **Roche Nominates First Selected GalXC™ Target.** Dicerna today announced that Roche has formally nominated the first selected target and thus has 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.
- **Dicerna and Alnylam Pharmaceuticals, Inc. Complete Cross-License Agreement for Primary Hyperoxaluria Programs and Form RNAi Therapeutics Collaboration on Alpha-1 Antitrypsin Deficiency-Associated Liver Disease.** In April 2020, Dicerna and Alnylam Pharmaceuticals, Inc. ("Alnylam") completed a cross-license of their respective intellectual property for Alnylam's lumasiran and Dicerna's nedosiran investigational programs for the treatment of PH. The cross-license agreement provides for Alnylam to pay mid- to high-single-digit royalties to Dicerna based on global net sales of lumasiran and for Dicerna to pay low-single-digit royalties to Alnylam on global net sales of nedosiran.

Dicerna and Alnylam also announced the formation of a development and commercialization collaboration for the treatment of alpha-1 antitrypsin ("A1AT") deficiency-associated liver disease. Under the agreement, Dicerna will evaluate Alnylam's ALN-AAT02 as well as Dicerna's DCR-A1AT, each investigational RNAi therapeutics in Phase 1/2 development for treatment of A1AT deficiency-associated liver disease, and Dicerna will select which product candidate to advance into pivotal development. At the completion of Phase 3, Alnylam may opt in to commercialize the selected candidate in countries outside the U.S.

- **Initial Observations From PHYOX3 Trial of Nedosiran for Treatment of Primary Hyperoxaluria Announced.** In March, Dicerna announced results from an interim analysis of the PHYOX3 long-term, multidose, open-label rollover extension trial of nedosiran. As of the March 2020 preliminary analysis:

- 14 participants from the completed single-dose PHYOX1 Phase 1 clinical trial had enrolled in the PHYOX3 trial;
- Four patients had received at least three monthly doses of nedosiran delivered subcutaneously; all four patients who received at least three monthly doses of nedosiran achieved normalization or near-normalization of urinary oxalate levels on at least two visits;
- Nedosiran appeared generally well tolerated, with an overall adverse event profile comparable to that observed in the PHYOX1 Phase 1 clinical trial;
- Based on the cumulative number of days patients had participated in the PHYOX3 trial, total patient exposure to monthly dosing of nedosiran delivered subcutaneously had reached nearly two years, and the longest-treated patient in the PHYOX3 trial had received seven monthly doses of nedosiran as of the interim analysis.

Due to the further postponement of the OxalEurope meeting to Dec. 1, 2020, the Company expects to first present multidose results from the PHYOX3 trial at its R&D Day planned for August 2020.

- **Orphan Drug Designation for DCR-A1AT.** In March, the U.S. Food and Drug Administration (FDA) granted orphan drug designation (ODD) to Dicerna's DCR-A1AT for the treatment of A1AT deficiency.

- **Eli Lilly & Company Selects Second Dicerna Molecule for Preclinical Evaluation.** During the first quarter of 2020, Eli Lilly & Company ("Lilly") selected LY3819469, a GalXC molecule for the second collaboration target in cardiometabolic disease, for advancement into preclinical development.

Clinical and Supply Chain Updates

In March, the Company provided a business and clinical development milestones update related to the COVID-19 pandemic. Given the fluid nature of the COVID-19 pandemic, the evolving and extraordinary actions undertaken by clinical trial sites globally, and the variable and uncertain pace at which clinical sites and territories may return to more conventional operations, Dicerna continues to evaluate the plans and timing related to its ongoing clinical development programs.

- **Nedosiran PHYOX Clinical Development Program**
  - **PHYOX2:** Enrollment in the PHYOX2 trial continues at a limited number of sites globally. As planned, Dicerna is implementing the necessary protocol amendments and is working closely with local Institutional Review Boards (IRBs) to facilitate the transition of certain site visits to a combination of at-home nurse visits with investigator telehealth assessments for drug administration and safety follow-up in the PHYOX2 trial. Patients continue to be screened for potential enrollment in the PHYOX2 trial, as feasible.

  - **PHYOX3:** As of April 2020, the Company had implemented a protocol amendment with local IRBs and had transitioned certain site visits to a combination of at-home nurse visits with investigator telehealth assessments for dose administration and safety follow-up. Patients have continued to enroll in the PHYOX3 trial, and as of May 4, 2020, 17 patients had enrolled in the study.

The Company plans to provide revised timing estimates for PHYOX2 enrollment completion and initiation of additional planned PHYOX trials at a later date and will continue to evaluate potential effects on timing of additional activities, such as the nedosiran New Drug Application submission.

- **DCR-A1AT Phase 1/2 Trial Update**

Following our business update in March 2020, enrollment of healthy volunteers in the Phase 1/2 trial of DCR-A1AT was effectively paused due to site restrictions related to the COVID-19 pandemic. All subjects in the current dosing cohort are expected to complete their remaining visits, as feasible. As of late April, the Scientific Review Committee for the DCR-A1AT Phase 1/2 trial confirmed that the study could continue and begin enrolling healthy volunteers in the next dosing cohort. Additional safety precautions will be implemented, including testing of any participants who present with symptoms consistent with COVID-19. The Company expects that participants will begin enrolling in the next dosing cohort in the next few weeks. Completion of dosing in healthy volunteers in the single-ascending-dose cohorts and initiation of dosing in patients in the Phase 1/2 trial of DCR-A1AT will be determined based on the timing and pace of enrollment, further developments in the COVID-19 pandemic, as well as the Company's evaluation of next steps for the ALN-AAT02 and DCR-A1AT programs under the agreement with Alnylam.

- **RG6346 Phase 1 Proof-of-Concept Trial for Hepatitis B Virus Infection**

The Phase 1 clinical trial of RG6346 for the treatment of chronic HBV infection continues to progress. Dicerna continues to expect to present preliminary Phase 1 proof-of-concept data from all existing cohorts at the Company's R&D Day in August 2020.

- **Supply Chain**

Supply of Dicerna's investigational medicines is sufficient to support ongoing clinical trials. Based on current evaluations, Dicerna's supply chains continue to appear intact at this time to meet the Company's foreseeable 2020 clinical, nonclinical and chemistry, manufacturing and control (CMC) 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 the needs of the Company and its collaborative partners 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.

Expected Upcoming Milestones and Events

- **R&D Day and Corporate Update** – August 2020
  - **Nedosiran**: Interim multidose data from PHYOX3 open-label clinical trial
  - **RG6346**: Preliminary Phase 1 proof-of-concept data from all existing cohorts
  - **GalXC**: Present data for extending GalXC technology to additional tissues
- **Nedosiran**: Updated multidose data from PHYOX3 open-label clinical trial – OxalEurope Meeting, Dec. 1, 2020
- **Collaborative Program**: Investigational New Drug or Clinical Trial Authorization filing for LY3561774 by Lilly – late 2020

Financial Condition and Operating Results for the First Quarter of 2020

- **Cash Position** – As of March 31, 2020, Dicerna had $706.9 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 March 31, 2020 and Dec. 31, 2019, respectively, reflecting collateral securing the Company's lease obligations.
- **Revenue** – Dicerna recognized $34.0 million of revenue associated with its collaboration agreements during the quarter ended March 31, 2020, compared to $3.1 million for the same period in 2019.
- **Research and Development (R&D) Expenses** – R&D expenses were $43.2 million for the quarter ended March 31, 2020, compared to $21.6 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 $16.0 million for the quarter ended March 31, 2020, compared to $9.7 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 $22.5 million, or $0.31 per share, for the quarter ended March 31, 2020, compared to $26.2 million, or $0.38 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.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's first quarter 2020 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 3780976 prior to the start of the call. The call will also be webcast 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 +1 (404) 537-3406 and refer to conference ID 3780976. The webcast will also be archived on Dicerna's website.

About Dicerna's GalXC™ RNAi Technology Platform

Dicerna's proprietary ribonucleic acid 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.

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 reducing the level of disease-causing genes of the liver, Dicerna's GalXC has the potential to safely target conditions that are difficult to treat with other modalities. Continually innovating, Dicerna is also exploring new applications of 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, cardiovascular, 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) the therapeutic and commercial potential of nedosiran, RG6346, ALN-AAT02, DCR-A1AT and the GalXC™ platform; (ii) clinical development timelines related to nedosiran, RG6346, ALN-AAT02 and DCR-A1AT, including continued alignment on the regulatory pathway to approval of nedosiran; and research and development plans relating to GalXC internal and partnered programs; (iii) the potential for Dicerna to continue 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 in our internal discovery research and in our collaborative programs; (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 investigational therapies, some of which are early-stage, such as nedosiran, RG6346, DCR-A1AT, our collaborative research and development programs 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 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 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 and the regulatory review of INDs and CTAs; 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 BALANCE SHEETS (In thousands)	**March 31, 2020**	**December 31, 2019**
Cash and cash equivalents	$245,479	$ 152,816
Held-to-maturity investments	461,411	196,065
Contract receivables	15,000	200,354
Prepaid expenses and other current assets	8,606	6,934
Property and equipment, net	7,500	7,076
Right-of-use operating assets, net	29,932	30,102
Restricted cash equivalents	5,563	3,894
Other noncurrent assets	5,298	168
Total Assets	$778,789	$ 597,409
Accounts payable	$ 6,497	$ 6,077
Accrued expenses and other current liabilities	19,087	20,042
Lease liability, current	3,164	3,358
Deferred revenue, current	223,556	212,258
Lease liability, noncurrent	20,518	20,141
Deferred revenue, noncurrent	327,506	182,730
Other noncurrent liabilities	555	608
Total stockholders' equity	177,906	152,195
Total Liabilities and Stockholders' Equity	$778,789	$ 597,409
Common stock outstanding	73,779	71,573

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended March 31, 2020**	**Three Months Ended March 31, 2019**
Revenue	$ 34,028	$ 3,107
Operating expenses:		
Research and development	43,171	21,603
General and administrative	16,023	9,676
Total operating expenses	59,194	31,279
Loss from operations	(25,166)	(28,172)
Other income (expense):		
Interest income	2,613	2,018
Interest expense	(4)	—

Other income	65	—
Total other income, net	2,674	2,018
Net loss	$ (22,492)	$ (26,154)
Net loss per share – basic and diluted	$ (0.31)	$ (0.38)
Weighted-average common shares outstanding – basic and diluted	72,919	68,259

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