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

November 5, 2020

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

– Company Reported $609.9 Million in Cash, Cash Equivalents and Marketable Securities as of September 30, 2020 –

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 5, 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 third quarter ended Sept. 30, 2020.

"Our mission to maximize the impact of RNAi on medicine and the strategy to accomplish this goal remain clear and actionable by advancing high-value internal programs and partnering with world-class biopharma companies in select therapeutic areas. We remain on track to complete enrollment of our PHYOX2 pivotal trial supporting nedosiran, our wholly owned product candidate for patients with primary hyperoxaluria, or PH, in the fourth quarter of 2020. Recent interim data from our ongoing PHYOX3 open-label trial of nedosiran instill great confidence in our organization that nedosiran may have a best-in-class profile as the first potential treatment for all known subtypes of PH and position us well as we transition into a fully integrated biopharmaceutical company," said Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. "Further supporting this transition is over $500 million in upfront payments secured through collaboration agreements over the last two years, which also carry tremendous future value through a stream of potential near- and long-term milestone payments, the first of which will be from our collaboration with Lilly in the fourth quarter of this year. Over the balance of 2020 and 2021, we anticipate receiving over $100 million in cash from our collaborations and look forward to updating investors as these partnerships mature and our internal pipeline expands."

Clinical and Regulatory Updates

- **Updated Positive Interim Data From Ongoing PHYOX3 Open-Label Trial Presented at the American Society of Nephrology (ASN) Annual Scientific Conference.** In October, the Company announced positive updated interim data as of Sept. 30, 2020 from its ongoing open-label extension study of nedosiran for the treatment of PH, a family of ultra-rare genetic disorders that initially manifest with complications in the kidneys through the overproduction of oxalate, which compromises kidney function and can have life-threatening consequences. All 13 participants (10 with PH1 and three with PH2) receiving nedosiran, who had previously completed the PHYOX1 Phase 1 trial and had reached Day 180 in the ongoing PHYOX3 trial, achieved normal (12 of 13) or near-normal (one of 13) urinary oxalate (Uox) excretions at one or more timepoints. Of these, all 10 (100%) of the participants with PH1, and two of the three (67%) participants with PH2, achieved normal Uox excretions at one or more visits. Nedosiran was generally well tolerated, and no serious safety concerns were identified in this ongoing study.

- **RG6346 Phase 1 Proof-of-Concept Trial for Chronic Hepatitis B Virus Infection**. In August, Dicerna announced positive interim data as of June 25, 2020 for RG6346, an investigational candidate for the treatment of chronic hepatitis B virus (HBV) infection being developed in collaboration with Roche. The data demonstrated a strong and durable reduction in hepatitis B surface antigen (HBsAg), a key viral marker of the disease, and a long and stable duration of activity in participants that received RG6346 with concomitant nucleos(t)ide (NUC) therapy. Nine of 10 participants in Group C who received either 1.5, 3.0 or 6.0 mg/kg of RG6346 plus NUC therapy and completed the treatment period at Day 112 achieved ≥1.0 log10 IU/mL reduction in HBsAg and continued into an ongoing extended follow-up period. Among Group C participants who completed the Day 112 treatment period, the maximum HBsAg reduction from baseline was 2.7 log10 IU/mL (3.0 mg/kg Group C cohort). In the longest-observed participant at the time of the data cut-off, the maximum HBsAg reduction from baseline was 2.2 log10 IU/mL (1.5 mg/kg Group C cohort) at Day 392 during the conditional follow-up period. As of the interim data analysis, six of 10 participants who received RG6346 plus NUC therapy and completed the Day 112 treatment period had HBsAg less than 100 IU/mL at the last reported visit. In the 3.0 and 6.0 mg/kg Group C cohorts, the mean HBsAg reduction at Day 112 was 1.8 log10 IU/mL or greater. No serious adverse events (SAEs) were observed with RG6346 treatment in any study group, and there were no dose-limiting toxicities or safety-related discontinuations. 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 in this study.

- **Preclinical Data From Dicerna's Technology in New Tissues.** In August, 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 showed consistent and durable CNS-wide target messenger RNA (mRNA) knockdown using novel constructs regardless of route of administration (intrathecal [IT] or intracisterna magna [ICM]) and reduction in target mRNA in skeletal muscle and adipose tissue using optimized chemistries, resulting in equivalent and potentially highly durable target knockdown regardless of dosing regimens.

Corporate Updates

- **Executive Appointments:** Dicerna strengthened its executive leadership team in the third quarter of 2020 with the appointments of Shreeram Aradhye, M.D., as executive vice president and chief medical officer and Ling Zeng as chief legal officer and secretary.

Anticipated Upcoming Milestones

- **Nedosiran:**
  - Enrollment completion of PHYOX2 pivotal trial in fourth quarter 2020
  - PHYOX2 last patient out first half 2021
  - New Drug Application submission third quarter 2021
- **Collaborative Program:** Investigational New Drug application or Clinical Trial Application for LY3561774 in late 2020
- **RG6346:** Updated Phase 1 trial data at The American Association for the Study of Liver Diseases 2020 scientific conference, Late-Breaking Oral Session 2 on Monday, Nov. 16, 2020 at 2:20 p.m. ET
- **DCR-A1AT/ALN-AAT02:** Program selection for advancement into Phase 2 in the first quarter 2021

Supply Chain Update

The current supply of Dicerna's investigational medicines continues to be sufficient to support ongoing and planned clinical trials. Based on current evaluations, we expect Dicerna's supply chain to meet the next 12 months of 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 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 Third Quarter of 2020

- **Cash Position** – As of Sept. 30, 2020, Dicerna had $609.9 million in cash, cash equivalents and held-to-maturity investments, compared to $348.9 million as of Dec. 31, 2019.
- **Revenue** – Dicerna recognized $48.9 million of revenue associated with its collaboration partners during the quarter ended Sept. 30, 2020, compared to $8.0 million for the same period in 2019.
- **Research and Development (R&D) Expenses** – R&D expenses were $54.8 million for the quarter ended Sept. 30, 2020, compared to $30.1 million for the same period in 2019. The increase was primarily due to direct research and development expenses and employee-related expenses associated with increased headcount necessary to support our collaboration agreements and expanding pipeline.
- **General and Administrative (G&A) Expenses** – G&A expenses were $17.0 million for the quarter ended Sept. 30, 2020, compared to $10.6 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 growing operations.
- **Net Loss** – Net loss was $21.8 million, or $0.29 per share, for the quarter ended Sept. 30, 2020, compared to $30.8 million, or $0.45 per share, for the same period in 2019.

Guidance

Dicerna believes that its cash, cash equivalents, held-to-maturity investments and anticipated milestone and other payments from existing collaborations will be sufficient to fund the execution of its current clinical and operating plan into 2023, which includes our expectations to advance nedosiran through planned pivotal development, regulatory filing and approval, commercial launch and supporting all R&D activities for current pipeline programs, both internal and collaboration. This estimate assumes no funding from new 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, largely due to investments in staffing and market readiness activities, clinical manufacturing activities, continued clinical activities associated with its core product candidates, and continued activities under its existing collaboration agreements.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's third 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 4263642 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 4263642. The webcast will also be archived on Dicerna's website.

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 silence selectively 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.

(tables follow)

DICERNA PHARMACEUTICALS, INC.

SELECTED FINANCIAL INFORMATION (UNAUDITED)

CONDENSED CONSOLIDATED BALANCE SHEETS (In thousands)	**September 30, 2020**	**December 31, 2019**
Cash and cash equivalents	$ 204,281	$ 152,816
Held-to-maturity investments	405,632	196,065
Contract receivables	5,028	200,354
Prepaid expenses and other current assets	12,710	6,934
Property and equipment, net	12,846	7,076
Right-of-use operating assets, net	28,136	30,102
Restricted cash equivalents	6,362	3,894
Other noncurrent assets	6,743	168
Total Assets	$ 681,738	$ 597,409
Accounts payable	$ 7,249	$ 6,077
Accrued expenses and other current liabilities	32,478	23,400
Deferred revenue, current	200,041	212,258
Deferred revenue, noncurrent	268,502	182,730
Other noncurrent liabilities	19,768	20,749
Total stockholders' equity	153,700	152,195
Total Liabilities and Stockholders' Equity	$ 681,738	$ 597,409
Common stock outstanding	74,681	71,573

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	Three Months Ended September 30, 2020	Three Months Ended September 30, 2019
Revenue	$ 48,875	$ 8,035
Operating expenses:		
Research and development	54,814	30,086
General and administrative	16,961	10,619
Total operating expenses	71,775	40,705
Loss from operations	(22,900)	(32,670)
Other income (expense):		
Interest income (expense), net	1,050	1,880
Other income	1	—
Total other income, net	1,051	1,880
Net loss	$ (21,849)	$ (30,790)
Net loss per share – basic and diluted	$ (0.29)	$ (0.45)
Weighted-average common shares outstanding – basic and diluted	74,523	68,360

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	Nine Months Ended September 30, 2020	Nine Months Ended September 30, 2019
Revenue	$ 123,351	$ 16,824
Operating expenses:		
Research and development	151,361	74,521
General and administrative	53,549	29,126
Total operating expenses	204,910	103,647
Loss from operations	(81,559)	(86,823)
Other income (expense):		
Interest income (expense), net	5,382	6,034
Other income	16	—
Total other income, net	5,398	6,034
Net loss	$ (76,161)	$ (80,789)
Net loss per share – basic and diluted	$ (1.03)	$ (1.18)
Weighted-average common shares outstanding – basic and diluted	73,817	68,315

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