Dicerna Announces Second Quarter 2021 Financial Results and Provides a Business Update

August 9, 2021

– Reported Positive Top-Line Data From Pivotal PHYOX ™2 Clinical Trial of Nedosiran Investigational GalXC ™ RNAi Therapy for Treatment of Primary Hyperoxaluria –

– Announced U.S. Food and Drug Administration (FDA) Clearance of Investigational New Drug (IND) Application for DCR-AUD for Alcohol Use Disorder –

– Announced Interim Phase 1 Results and Initiated Patient Dosing in ESTRELLA Phase 2 Study of Belcesiran for the Treatment of Alpha-1 Antitrypsin Deficiency-Associated Liver Disease (AATLD) –

– Received $10.0 Million Milestone Payment Following FDA Acceptance of Lilly's Second GalXC RNAi IND Application Under Companies' Global Research Collaboration and Licensing Agreement –

– Company Reported $709.6 Million in Cash, Cash Equivalents and Held-to-Maturity Investments as of June 30, 2021 –

– Management to Host Conference Call Today at 8:00 a.m. ET –

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 9, 2021-- 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 quarter ended June 30, 2021 and provided a corporate update.

"We have seen significant progress across our core and collaborative pipelines over the past few months, including the achievement of a major milestone in our maturation as a company," said Douglas Fambrough, Ph.D., President and Chief Executive Officer at Dicerna. "Specifically, our recently announced PHYOX™2 results represent our first positive pivotal trial readout. PHYOX2 generated robust data, meeting the primary and key secondary efficacy endpoints, and nedosiran was generally well tolerated in the trial. Based on these results, we believe nedosiran has significant potential as a treatment for patients with PH1, and we are on course to submit a New Drug Application to the FDA for the treatment of PH1 in the fourth quarter. Notably, these positive results reflect broadly on our GalXC™ RNAi platform and bode well for our additional proprietary and partnered programs.

"As positive as these results are for PH1, the inconsistent data seen specifically in participants with PH2 have led us to make the strategic decision not to move forward with our plan to build Dicerna into a fully integrated commercial enterprise to support nedosiran," Dr. Fambrough continued. "Instead, we intend to pursue commercial out-licensing opportunities to help ensure global access to nedosiran, subject to necessary approvals. This approach will allow us to deploy our capital and talent on our discovery and development pipeline efforts with our GalXC and GalXC-Plus™ RNAi investigational therapeutics for ourselves and our partners. With these strategic adjustments focused on our core strengths, we can extend our cash runway into 2025."

Recent Updates

- **Positive Top-Line Data From Pivotal PHYOX2 Clinical Trial of Nedosiran for Primary Hyperoxaluria (PH).** Dicerna announced in August positive top-line results from the pivotal PHYOX2 clinical trial of nedosiran. Nedosiran achieved the primary endpoint in the PHYOX2 trial, demonstrating a statistically significant reduction from baseline in urinary oxalate (Uox) excretion compared to placebo ($p<0.0001$). The study also achieved the key secondary endpoint, with a significantly higher proportion of patients given nedosiran achieving and sustaining normal or near-normal Uox at two or more consecutive visits after Day 90 compared to placebo ($p=0.0025$). Uox reductions were significant in participants with PH1 while participants with PH2 (5 nedosiran and 1 placebo) showed inconsistent results in this trial. Nedosiran was generally well tolerated in the study with an overall adverse event (AE) profile consistent with previously reported data from PHYOX trials. The Company expects the results from the PHYOX2 trial to support marketing authorization applications for the treatment of PH1 in the U.S. and other major markets and intends to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2021.

- **Announced Clearance of Investigational New Drug (IND) Application for DCR-AUD for the Treatment of Alcohol Use Disorder (AUD).** In July 2021, Dicerna announced U.S. Food and Drug Administration (FDA) clearance of the IND application for DCR-AUD, the Company's investigational GalXC RNAi candidate for the treatment of AUD. Dicerna plans to initiate a Phase 1 trial in the third quarter of 2021 to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single ascending doses of DCR-AUD in healthy volunteers.

- **Reported Interim Data From Phase 1 Trial of Belcesiran.** In July 2021, Dicerna reported interim data from the Company's Phase 1 trial of belcesiran, a GalXC RNAi therapeutic candidate in development for the treatment of alpha-1 antitrypsin (AAT) deficiency-associated liver disease (AATLD). The trial is designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of belcesiran in healthy volunteers. Data from this interim analysis of the four completed active-treatment dose cohorts (0.1, 1.0, 3.0 and 6.0 mg/kg) showed dose-dependent reductions in serum AAT with administration of a single dose of belcesiran. In this analysis, belcesiran was found to have an acceptable safety

profile and was generally well tolerated. The final 12.0 mg/kg dose cohort in this trial is ongoing, and the Company plans to present additional data from this study at a medical congress in 2021, subject to abstract acceptance.

- **Initiated ESTRELLA Phase 2 Clinical Trial Patient Dosing of Belcesiran for the Treatment of AATLD.** In June 2021, Dicerna announced initiation of the Company's ESTRELLA Phase 2 trial for belcesiran, as part of the SHINE clinical development program for the treatment of AATLD. The ESTRELLA Phase 2 trial is a randomized, multidose, double-blind, placebo-controlled study evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of belcesiran in participants with AATLD.

- **Completed Nedosiran PHYOX4 Clinical Trial Dosing for Treatment of Primary Hyperoxaluria Type 3.** In June 2021, Dicerna announced completion of patient dosing in its PHYOX4 trial, a randomized, placebo-controlled, double-blind, multicenter study evaluating safety and tolerability of nedosiran for the treatment of PH3. PHYOX4 is a part of the PHYOX clinical development program evaluating nedosiran in patients with all three known subtypes of PH. The Company expects to report top-line results from this study in October 2021.

Collaboration Updates

- **Reported FDA Acceptance of IND for LY3819496 Filed by Eli Lilly and Company ("Lilly").** In May 2021, Dicerna announced FDA acceptance of Lilly's IND for LY3819469, the second clinical-stage investigational GalXC RNAi candidate to emerge from Dicerna's collaboration with Lilly. The IND milestone triggered a $10.0 million payment to Dicerna, and in June 2021, Lilly initiated dosing in a Phase 1 clinical trial of LY3819469, targeting the *LPA* gene, as a potential treatment of cardiometabolic diseases.

- **Announced Boehringer Ingelheim's Acceptance of DCR-LIV2 for Development Under RNAi Research Collaboration and License Agreement.** In May 2021, Dicerna announced Boehringer Ingelheim's (BI) acceptance of DCR-LIV2, an investigational GalXC RNAi candidate for advancement under the existing agreement between the companies for the discovery and development of novel therapies for the treatment of chronic liver diseases. DCR-LIV2 will be evaluated for the treatment of nonalcoholic steatohepatitis (NASH), a chronic liver disease for which there are no approved therapeutic interventions. This candidate acceptance triggered a single-digit multimillion-dollar preclinical milestone payment to Dicerna in the second quarter of 2021.

Anticipated Upcoming 2021 Milestones

- **Nedosiran:**
  - Top-line data from PHYOX4 trial in patients with PH3 in October 2021
  - Initiate PHYOX8 trial, an open-label study in patients aged 0-5 years with PH1 or PH2, in the third quarter of 2021
  - NDA submission in the fourth quarter of 2021

- **Belcesiran:** Present Phase 1 data at a medical congress, subject to abstract acceptance

- **DCR-AUD:** Initiate Phase 1 study in healthy volunteers in the third quarter of 2021

Financial Results for the Second Quarter Ended June 30, 2021

- **Cash Position** – As of June 30, 2021, Dicerna had $709.6 million in cash, cash equivalents and held-to-maturity investments, compared to $568.8 million as of Dec. 31, 2020.

- **Revenue** – Dicerna recognized $41.3 million of revenue for the second quarter 2021, compared to $40.4 million for the same period in 2020. Revenue was relatively flat for the second quarter 2021, compared to the same period in 2020, as increases in Lilly, Novo, and BI revenues were largely offset by decreases in Roche and Alexion revenues.

- **Research and Development (R&D) Expenses** – R&D expenses were $56.1 million for the second quarter 2021, compared to $53.4 million for the same period in 2020. The increase was primarily due to increases in facilities, depreciation, and other expenses and employee-related expenses as a result of an increase in R&D headcount necessary to support the Company's expanding pipeline and collaboration agreements. These increases were largely offset by a decrease in direct R&D expenses, primarily due to decreases in drug substance expense.

- **General and Administrative (G&A) Expenses** – G&A expenses were $25.5 million for the second quarter 2021, compared to $20.6 million for the same period in 2020. The increase was primarily due to an increase in professional consulting fees.

- **Net Loss** – Net loss was $40.8 million, or $0.53 per share, for the second quarter ended June 30, 2021, compared to a net loss of $31.8 million, or $0.43 per share, for the same period in 2020.

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 2025, which includes supporting all R&D activities for current internal and collaboration pipeline programs. 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 research and development expenses to continue to increase for the foreseeable future, largely due to clinical manufacturing activities, continued clinical activities associated with its core product candidates and continued activities under its existing collaboration agreements. The Company continues to forecast receiving $83.0 million in cash from its current collaboration agreements during full-year 2021, of which $74.5 million has been received in the first six months of 2021.

Conference Call

Management will host a conference call at 8:00 a.m. ET today to review Dicerna's second quarter 2021 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 5418088 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 5418088. 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 GalXC™ and GalXC-Plus™ RNAi technologies, 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 disease-causing genes in the liver, Dicerna has continued to innovate and is exploring new applications of its RNAi technology with GalXC-Plus, which expands the functionality and application of our flagship liver-targeted GalXC technology to tissues and cell types outside the liver, and has the potential to treat diseases across multiple therapeutic areas. 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 cardiometabolic, viral, chronic liver and complement-mediated diseases, as well as neurodegenerative diseases and pain. At Dicerna, our mission is to interfere – to silence genes, to fight disease, to restore health. For more information, 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 our and our collaborative partners’ product candidates and the development thereof, including nedosiran, belcesiran, DCR-AUD, DCR-LIV2 and LY3819469; the progress of and anticipated milestones for the Company’s ongoing and planned trials, including those from its PHYOX program as well as other trials of nedosiran; results from ongoing and planned trials of the Company’s PHYOX clinical development program; the initiation of trials for product candidates in our pipeline, including nedosiran and DCR-AUD; the filing of INDs of our and our partners’ product candidates; the therapeutic potential of our product candidates, including nedosiran; the planned submission of the New Drug Application (NDA) for nedosiran and our commercialization strategy for nedosiran, if approved; our collaborations and other strategic arrangements, including the intended benefits thereof; our business and operations, including the discovery, development and commercialization of our product candidates and technology platform, and the therapeutic potential thereof; our collaborations with partners, including the pace and progress of development by our collaboration partners, the receipt of anticipated milestone payments therefrom, any potential future collaborations; and our financial position and cash runway.

The process by which investigational therapies, such as nedosiran and belcesiran, 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; 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, development and commercialization and the risk of supply interruption from any contract manufacturer; the potential for future data to alter initial and preliminary results of preclinical studies, models and earlier-stage clinical trials; the impact of the ongoing COVID-19 pandemic and its variants 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 (IND) applications 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 NDAs and comparable foreign applications for one or more of Dicerna’s product candidates, including for nedosiran; alignment with the FDA on the regulatory pathway to approval for our product candidates, including nedosiran; the ability to secure out-licensing opportunities to commercialize nedosiran, if approved, in the U.S. and abroad on acceptable terms, if at all; 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 following commercialization; changes in our current clinical and operating plan; 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)	**June 30, 2021**	**December 31, 2020**
Cash and cash equivalents	$221,210	$ 126,023
Held-to-maturity investments	488,354	442,820
Restricted cash equivalents	5,618	6,362
Contract receivables	3,147	34,713
Prepaid expenses and other current assets	20,380	14,403
Property and equipment, net	23,593	17,546
Right-of-use operating assets, net	74,400	60,843
Other noncurrent assets	1,790	5,136
Total Assets	$838,492	$ 707,846
Accounts payable	$ 12,829	$ 7,901
Accrued expenses and other current liabilities	33,523	31,500
Deferred revenue, current	160,200	138,537
Deferred revenue, noncurrent	268,606	336,236
Deferred income	179,806	—
Other noncurrent liabilities	72,454	55,918
Total stockholders' equity	111,074	137,754
Total Liabilities and Stockholders' Equity	$838,492	$ 707,846
Common stock outstanding	77,602	75,757

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended June 30, 2021**	**Three Months Ended June 30, 2020**
Revenue	$ 41,337	$ 40,448
Operating expenses:		
Research and development	56,119	53,376
General and administrative	25,462	20,565
Total operating expenses	81,581	73,941
Loss from operations	(40,244)	(33,493)
Other income (expense):		
Interest income, net	107	1,723
Other expense	(132)	(50)
Total other (expense) income	(25)	1,673
Loss before income taxes	(40,269)	(31,820)
Provision for income taxes	(546)	—
Net loss	$ (40,815)	$ (31,820)
Net loss per share – basic and diluted	$ (0.53)	$ (0.43)
Weighted average common shares outstanding – basic and diluted	77,030	74,001

View source version on businesswire.com: https://www.businesswire.com/news/home/20210809005221/en/

Media:
Amy Trevvett
+1 617-612-6253
atrevvett@dicerna.com

Investors:
Janhavi Mohite
+1 212-362-1200
ir@dicerna.com

Source: Dicerna Pharmaceuticals, Inc.