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

November 9, 2021

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

– Initiated Phase 1 Clinical Trial of DCR-AUD for the Treatment of Alcohol Use Disorder (AUD) –

– Company Plans to Unveil First GalXC-Plus™ Extrahepatic Target in Early 2022 –

– Cash Runway Extends Into 2025 –

– Company to Host Conference Call Today at 8:30 a.m. ET –

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 9, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported its financial results for the third quarter ended September 30, 2021 and provided a business update.

"We had an exciting and productive third quarter as we continued to execute and advance key initiatives that we believe help position Dicerna for multiple value-creating milestones over the next 12 to 24 months," said Douglas Fambrough, Ph.D., President and Chief Executive Officer at Dicerna. "We look to build on this progress as we continue advancing core programs that include nedosiran for primary hyperoxaluria (PH), RG6346 for chronic hepatitis B virus (HBV) infection with Roche, belcesiran for alpha-1 antitrypsin deficiency-associated liver disease (AATLD) and DCR-AUD for alcohol use disorder (AUD). We also look forward to unveiling the next wave of innovations from Dicerna in early 2022 with the first of several wholly owned extrahepatic programs harnessing our GalXC-Plus technology."

Dr. Fambrough continued, "Between Dicerna and our collaborative partners, we have 16 programs in preclinical or clinical development as well as more than 20 discovery-stage programs. Given the breadth of these activities, we expect new programs to be entering the clinic, on average, one per quarter over the next two years and potentially beyond that at a similar rate. We believe this creates the opportunity for Dicerna to dramatically expand in the future and meaningfully advance our mission to improve the lives of patients."

Corporate Highlights

- **Announced Positive Top-Line Data From Pivotal PHYOX2 Clinical Trial of Nedosiran for Primary Hyperoxaluria.** In August 2021, Dicerna announced positive top-line results from the pivotal PHYOX2 clinical trial of nedosiran, Dicerna's late-stage investigational GalXC RNAi therapeutic candidate in development for PH. 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 PH type 2 (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.
- **Presented PHYOX2 Data as Late-Breaker Poster Presentation at American Society of Nephrology (ASN) Kidney Week 2021.** The data from Dicerna's pivotal PHYOX2 study were presented at ASN on November 4, 2021.
- **Reported Top-Line Results for PHYOX4 Single-Dose Study of Nedosiran in Primary Hyperoxaluria Type 3 (PH3).** In October 2021, Dicerna announced that its PHYOX4 study designed to evaluate the safety and tolerability of a single subcutaneous dose of nedosiran compared to placebo in patients with PH3 met its primary safety endpoint. Patients administered nedosiran also showed a trend in Uox reduction; however, these reductions did not meet prespecified secondary efficacy endpoint criteria.
- **Initiated Phase 1 Clinical Trial of DCR-AUD for the Treatment of Alcohol Use Disorder.** In September 2021, Dicerna announced that it dosed the first subjects in its Phase 1 clinical trial to assess DCR-AUD, Dicerna's investigational GalXC RNAi therapeutic candidate in development for the treatment of AUD. The randomized, double-blind study is evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of single ascending doses of DCR-AUD in up to 36 healthy volunteers over a 24-week observation period. The trial is also assessing the interaction between DCR-AUD administration and alcohol consumption using standardized Ethanol Interaction Assessments (EIA) performed serially over the trial's duration.
- **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 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) demonstrated 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. Dosing in the final

12.0 mg/kg dose cohort in this trial has been completed, and the Company plans to present data from this trial at the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® taking place Nov. 12-15, 2021.

Anticipated Upcoming Milestones

- **Nedosiran:** Subject to ongoing pre-NDA interactions with the U.S. Food and Drug Administration (FDA), submission of nedosiran New Drug Application (NDA) to FDA for the treatment of PH1 expected in the first quarter of 2022.
- **Belcesiran:** Phase 1 data to be presented at AASLD, Nov. 12-15, 2021.
- **DCR-AUD:** Interim Phase 1 data expected later in 2022.
- **GalXC-Plus:** First Dicerna GalXC-Plus extrahepatic clinical development program expected to be unveiled in early 2022.

Financial Results for the Third Quarter Ended September 30, 2021

- **Cash Position** – As of September 30, 2021, Dicerna had $646.6 million in cash, cash equivalents and held-to-maturity investments, compared to $568.8 million as of December 31, 2020.
- **Revenue** – Dicerna recognized $63.0 million of revenue for the third quarter 2021, compared to $48.9 million for the same period in 2020. The increase in revenue for the third quarter of 2021 primarily reflects an increase in revenue from the Company's collaboration with Novo Nordisk A/S.
- **Research and Development (R&D) Expenses** – R&D expenses were $61.2 million for the third quarter 2021, compared to $54.8 million for the same period in 2020. The increase was primarily due to higher facility-related expenses as well as increased depreciation and other expenses.
- **General and Administrative (G&A) Expenses** – G&A expenses were $22.0 million for the third quarter 2021, compared to $17.0 million for the same period in 2020. The increase was primarily due to increased rent expenses and an increase in software costs.
- **Net Loss** – Net loss was $17.1 million, or $0.22 per share, for the third quarter 2021, compared to a net loss of $21.8 million, or $0.29 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 R&D 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 $76.5 million has been received in the first nine months of 2021.

Conference Call

Management will host a conference call at 8:30 a.m. ET today to review Dicerna's third 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 7395414 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 7395414. 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 GalXC and GalXC-Plus RNAi technologies and the therapeutic potential thereof, including the advancement of our new, wholly owned GalXC-Plus extrahepatic development programs and timing to announce the first of them; our expected pace and duration of new programs entering into clinical trials and the potential impact thereof; our discovery and product candidates and those of our collaborative partners,

including with respect to nedosiran, belcesiran and DCR-AUD, and the development thereof; the pace and progress of and anticipated milestones for the Company's ongoing and planned trials, including from its Phase 1 clinical trial of DCR-AUD for the treatment of AUD; results and expectations from the Company's ongoing and completed trials, including from the Company's PHYOX clinical development program; the initiation of trials for product candidates in our pipeline and our beliefs about our pipeline; the filing of Investigational New Drug Applications (INDs) for our product candidates and those of our collaboration partners, including the expected pace thereof; the therapeutic potential of our product candidates, including nedosiran; the planned submission of an NDA for nedosiran and the expected timing thereof, subject to ongoing interactions with the FDA; our commercialization strategy for nedosiran, if approved; our current and potential future collaborations and other strategic arrangements, including the intended benefits thereof, pace and progress of development by such partners and the receipt of anticipated milestone payments therefrom; our strategy, business and operations; and our financial position and expected 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 preclinical and 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 manufacturing organizations 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 process and unpredictability of the duration and results of the regulatory review of IND applications and clinical trial applications 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 for 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 our collaborations and other strategic partners; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain or secure, and costs to obtain, secure and maintain 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 strategy, business or 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)	**SEPTEMBER 30, 2021**	**DECEMBER 31, 2020**
Cash and cash equivalents	$ 165,756	$ 126,023
Held-to-maturity investments	480,837	442,820
Restricted cash equivalents	5,618	6,362
Contract receivables	6,226	34,713
Prepaid expenses and other current assets	17,268	14,403
Property and equipment, net	22,096	17,546
Right-of-use operating assets, net	72,432	60,843
Other noncurrent assets	1,826	5,136
Total Assets	$ 772,059	$ 707,846
Accounts payable	$ 8,320	$ 7,901
Accrued expenses and other current liabilities	39,020	31,500
Deferred revenue, current	190,128	138,537
Deferred revenue, noncurrent	180,790	336,236
Deferred income	179,629	—
Other noncurrent liabilities	66,509	55,918
Total stockholders' equity	107,663	137,754
Total Liabilities and Stockholders' Equity	$ 772,059	$ 707,846
Common stock outstanding	77,835	75,757

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended September 30, 2021**	**Three Months Ended September 30, 2020**
Revenue	$ 62,954	$ 48,875
Operating expenses:		
Research and development	61,232	54,814
General and administrative	21,994	16,961
Total operating expenses	83,226	71,775

Loss from operations	(20,272)	(22,900)
Other income:		
Interest income, net	124	1,050
Other income, net	3,886	1
Total other income, net	4,010	1,051
Loss before income taxes	(16,262)	(21,849)
Provision for income taxes	(810)	—
Net loss	$ (17,072)	$ (21,849)
Net loss per share – basic and diluted	$ (0.22)	$ (0.29)
Weighted average common shares outstanding – basic and diluted	77,747	74,523

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

Investors:
Kristen K. Sheppard, Esq.
+ 1 617-514-2275
ksheppard@dicerna.com

Source: Dicerna Pharmaceuticals, Inc.