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

May 6, 2021

– Announced Sale of OXLUMO™ (lumasiran) Royalty Interest to Royalty Pharma for Up to $240 Million; Extends Cash Runway Into 2024 –

– Earned $25 Million Milestone Payment in Connection With Roche's Initiation of GalXC™ RNAi Candidate RG6346 in Phase 2 Combination Trial for Treatment of Chronic Hepatitis B Virus Infection –

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

LEXINGTON, Mass.--(BUSINESS WIRE)--May 6, 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 first quarter ended March 31, 2021 and provided a corporate update.

"We continue to execute across our expanding clinical pipeline, particularly across our PHYOX™ development program for nedosiran, where we anticipate the data from our PHYOX2 pivotal trial in patients with PH1 or PH2 sometime mid-year. We also recently dosed our first patient in PHYOX7, a multidose trial in patients with PH1 or PH2 who have severe renal impairment," said Douglas Fambrough, Ph.D., President and Chief Executive Officer of Dicerna. "Although enrollment in our PHYOX4 single-dose trial in patients with PH3 has been slower than anticipated in the current COVID-19 environment and will push our NDA submission from late in the third quarter to sometime in the fourth quarter, we expect that nedosiran's profile may offer compelling advantages in PH1 and be a significant, and the only, therapy for people with PH2 or PH3 who currently have no treatment options to impact disease progression."

Dr. Fambrough continued, "With regard to our other ongoing clinical programs, we anticipate filing an IND for DCR-AUD for the treatment of alcohol use disorder and expect top-line Phase 1 data in healthy volunteers for belcesiran, our investigational GalXC™ RNAi candidate for the treatment of alpha-1 antitrypsin deficiency-associated liver disease, around mid-year. We have initiated the belcesiran Phase 2 trial and look forward to announcing the initiation of dosing in the coming months as additional sites are activated. With the additional influx of $180 million in non-dilutive cash from the monetization of our lumasiran royalty, we hope to meet our goal of net-zero cash burn in 2021. With this additional cash, our runway has extended into 2024, which includes fully funding our planned development efforts across our pipeline and executing for a strong nedosiran commercial launch."

Recent Updates

- **Sold OXLUMO™ (lumasiran) Royalty Interest to Royalty Pharma for Up to $240 Million.** In April 2021, Royalty Pharma plc agreed to acquire Dicerna's royalty interest in OXLUMO™ (lumasiran) for an upfront cash payment of $180 million and up to $60 million in contingent sales-based milestone payments. OXLUMO, which has been approved by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) for the treatment of primary hyperoxaluria (PH) type 1, is marketed by Alnylam Pharmaceuticals, Inc. ("Alnylam").

- **Announced Roche's Initiation of GalXC™ RNAi Candidate RG6346 in Phase 2 Combination Trial for Treatment of Chronic Hepatitis B Virus (HBV) Infection.** In March 2021, Dicerna announced that Roche initiated RG6346 in a Roche-sponsored Phase 2 combination trial for the treatment of chronic HBV. RG6346 is an investigational GalXC RNAi therapeutic that Dicerna is developing in collaboration with Roche as part of the companies' worldwide collaboration and licensing agreement for chronic HBV treatments. The Phase 2 platform trial will evaluate the efficacy and safety of RG6346 in combination with multiple additional agents with different mechanisms of action. Dicerna earned a $25 million milestone in connection with the initiation of RG6346 in the Phase 2 combination trial.

- **Presented New Data From Preclinical Studies Demonstrating That GalXC-Plus™ RNAi Technology Delivers Target Knockdown Across Central Nervous System (CNS) and to Specific CNS Cell Types.** In March 2021, Dicerna presented new data from preclinical studies of its GalXC-Plus RNAi technology demonstrating its potential to deliver deep and sustained messenger RNA (mRNA) knockdown against prespecified gene targets across the CNS and to oligodendrocytes, astrocytes and neurons. The data were presented as part of the sixth annual Oligonucleotide and Precision Therapeutics (OPT) Virtual Congress.

Anticipated Upcoming 2021 Milestones

- **Nedosiran:**
  - Top-line data from PHYOX2 pivotal trial in patients with PH1 or PH2 mid-year 2021
  - Top-line data from PHYOX4 trial in patients with PH3 in the third quarter of 2021
  - Initiate PHYOX8 trial, an open-label study in patients aged 0-5 years with PH1 or PH2, in the second quarter of 2021
  - New Drug Application (NDA) submission in the fourth quarter of 2021

- **Belcesiran:** Initial top-line Phase 1 trial data mid-year 2021 and additional data at a scientific conference in the second half of 2021

- **DCR-AUD:** File investigational new drug (IND) application mid-year 2021 and initiate Phase 1 study in healthy volunteers in the third quarter of 2021
- **Collaborative Program:** Lilly IND for GalXC RNAi candidate LY3819469 targeting the *LPA* gene in the second quarter of 2021

Financial Results for the First Quarter Ended March 31, 2021

- **Cash Position** – As of March 31, 2021, Dicerna had $544.9 million in cash, cash equivalents and held-to-maturity investments, compared to $568.8 million as of Dec. 31, 2020. The cash position as of March 31, 2021 did not include proceeds from the sale of the OXLUMO royalty interest.
- **Revenue** – Dicerna recognized $47.6 million for the first quarter 2021, compared to $34.0 million for the same period in 2020. The increase in revenue on a year-over-year basis was primarily attributable to an increase in services performed under the collaboration agreement with Roche, as well as under the Alexion and Novo collaboration agreements.
- **Research and Development (R&D) Expenses** – R&D expenses were $56.0 million for the first quarter 2021, compared to $43.2 million for the same period in 2020. The increase was primarily due to an increase in employee-related expenses as a result of an increase in R&D headcount necessary to support the Company's expanding pipeline and collaboration agreements and an increase in direct R&D expenses for nedosiran.
- **General and Administrative (G&A) Expenses** – G&A expenses were $20.7 million for the first quarter 2021, compared to $16.0 million for the same period in 2020. The increase was primarily due to employee-related expenses as a result of increased headcount necessary to support the Company's growing operations and an increase in professional consulting fees.
- **Net Loss** – Net loss was $30.0 million, or $0.39 per share, for the first quarter ended March 31, 2021, compared to a net loss of $22.5 million, or $0.31 per share, for the same period in 2020.

Guidance

Dicerna believes that its cash, cash equivalents, held-to-maturity investments, the proceeds from the April 2021 royalty financing transaction and anticipated milestone and other payments from existing collaborations will be sufficient to fund the execution of its current clinical and operating plan into 2024, which includes the Company's expectations to advance nedosiran through planned pivotal development, regulatory filing and approval and commercial launch; and 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 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. The Company continues to forecast receiving over $83 million in cash from its current collaboration agreements during full-year 2021.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's first 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 8088867 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 8088867. 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 on the functionality and application of our flagship liver-targeted GalXC technology 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, 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 our and our collaborative partners' product candidates and the development thereof, including nedosiran, RG6346, belcesiran, DCR-AUD 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 RG6346, belcesiran and DCR-AUD, the filing of INDs of our and our partners' product candidates, including DCR-AUD and LY3819469, the therapeutic potential of our product candidates, including nedosiran, the planned submission of the New Drug Application (NDA) for nedosiran, our collaborations and other strategic arrangements, including the intended benefits of our lumasiran royalty interest sale, as well as 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 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, development and commercialization 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 (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; continued alignment with the FDA on the regulatory pathway to approval for nedosiran; 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; 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)	**MARCH 31, 2021**	**DECEMBER 31, 2020**
Cash and cash equivalents	$ 159,111	$ 126,023
Held-to-maturity investments	385,773	442,820
Restricted cash equivalents, current	—	744
Contract receivables	31,267	34,713
Prepaid expenses and other current assets	18,349	14,403
Property and equipment, net	18,873	17,546
Right-of-use operating assets, net	59,030	60,843
Restricted cash equivalents, noncurrent	5,618	5,618
Other noncurrent assets	5,389	5,136
Total Assets	$ 683,410	$ 707,846
Accounts payable	$ 11,276	$ 7,901
Accrued expenses and other current liabilities	32,917	31,500
Deferred revenue, current	137,954	138,537
Deferred revenue, noncurrent	318,318	336,236
Other noncurrent liabilities	56,541	55,918
Total stockholders' equity	126,404	137,754
Total Liabilities and Stockholders' Equity	$ 683,410	$ 707,846
Common stock outstanding	76,584	75,757

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended March 31, 2021**	**Three Months Ended March 31, 2020**
Revenue	$ 47,603	$ 34,028
Operating expenses:		
Research and development	56,038	43,171
General and administrative	20,672	16,023
Total operating expenses	76,710	59,194
Loss from operations	(29,107)	(25,166)
Other income (expense):		

Interest income, net		276		2,609
Other (expense) income		(1,134)		65
Total other (expense) income		(858)		2,674
Net loss	$	(29,965)	$	(22,492)
Net loss per share – basic and diluted	$	(0.39)	$	(0.31)
Weighted average common shares outstanding – basic and diluted		76,256		72,919

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