Dicerna Announces Full Year 2020 Financial Results and Provides a Business Update

February 25, 2021

– 2020 Highlights Include: Enrollment Completion of Pivotal PHYOX™2 Trial of Nedosiran for Primary Hyperoxaluria; Positive RG6346 Phase 1 Data for HBV; Positive Nedosiran Multidose Data from PHYOX3 Trial; First IND From Lilly Collaboration –

– Company Announces Internal Clinical Development Program for Treatment of Alcohol Use Disorder (AUD) With Mid-Year 2021 IND Filing Goal –

– Multiple Clinical and Collaboration Milestones Expected in 2021, Including Planned New Drug Application Submission for Nedosiran –

– $568.8 Million in Cash, Cash Equivalents and Marketable Securities as of December 31, 2020 –

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Feb. 25, 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 full year ended Dec. 31, 2020 and provided a corporate update.

“Despite pandemic headwinds, 2020 was an incredibly productive year for Dicerna as we grew and advanced our pipeline of GalXC™ RNAi therapeutics while expanding our reach to include diverse tissues around the body. Led by pivotal-stage nedosiran for primary hyperoxaluria, belcesiran for alpha-1 antitrypsin deficiency-associated liver disease, RG6346 for chronic HBV and *ANGPTL3*-targeted LY3561774 for dyslipidemia, we expect the clinical-stage pipeline of GalXC and GalXC-Plus™ drug development candidates to grow by two more programs by mid-year 2021 and at least four more by the end of 2022. We also expect a similar number of preclinical development programs targeting diverse tissues moving forward. Meanwhile, our balance sheet is strong, and our collaborative partnerships are expected to continue contributing non-dilutive funding to support our burgeoning clinical development efforts over the coming years,” said Douglas Fambrough, Ph.D., President and Chief Executive Officer of Dicerna. “Of particular note, we look forward to Roche’s near-term planned initiation of RG6346 in a Phase 2 combination trial for HBV, the initiation of our Phase 2 trial of belcesiran and top-line data from our PHYOX2 pivotal trial of nedosiran approximately mid-year.”

Dr. Fambrough continued, “We are excited to announce today our latest clinical development program: DCR-AUD is our investigational GalXC RNAi therapy for the treatment of alcohol use disorder (AUD), for which we plan to file an IND mid-year. Alcohol misuse is widespread with few pharmacological interventions. We believe the properties of our RNAi technology have the potential to transform AUD treatment by developing, for the first time, a potentially long-acting, well-tolerated, conveniently subcutaneously delivered, highly targeted therapeutic candidate to inhibit ALDH2, a key enzyme in alcohol metabolism. With these properties, we believe DCR-AUD could play an important role as a therapeutic complement to non-pharmaceutical behavioral approaches.”

“Alcohol use disorder is a serious and grossly undertreated condition, affecting an estimated 14 million U.S. adults in a one-year period. It is associated with nearly 100,000 deaths annually, making it one of the leading preventable causes of death in the United States,” said Henry Kranzler, M.D., Professor of Psychiatry and Director of the Center for Studies of Addiction at the University of Pennsylvania’s Perelman School of Medicine. “I applaud Dicerna’s initiative to develop a new and innovative pharmacological treatment as a supportive therapy for people struggling with alcohol use disorder and look forward to following the company’s progress as it moves DCR-AUD into clinical development.”

Recent Updates

- **Clinical Development Candidate DCR-AUD for Alcohol Use Disorder (AUD) To Enter Clinical Development in the Third Quarter of 2021.** Today, Dicerna announced its third wholly owned clinical development program, an investigational therapy based on Dicerna’s GalXC technology for the treatment of AUD. In animal models, DCR-AUD specifically knocks down *ALDH2* gene expression in the liver, which plays a key role in alcohol metabolism. Inhibition of *ALDH2* may help individuals with AUD avoid harmful levels of alcohol use.

  AUD is a chronic condition characterized by compulsive alcohol use, loss of control over alcohol use and a negative emotional state when not using alcohol. A range of medical, psychological, social, economic and personal problems are associated with AUD. Dicerna plans to file an Investigational New Drug Application (IND) or Clinical Trial Application (CTA) for DCR-AUD in mid-2021 and initiate a Phase 1 single-ascending-dose trial in healthy volunteers in the third quarter of 2021.

- **Initiated PHYOX4 Trial of Nedosiran for Primary Hyperoxaluria (PH) Type 3 (PH3).** In February 2021, the Company announced initiation of patient dosing in the Company’s PHYOX4 clinical trial to assess its lead investigational candidate, nedosiran, for the treatment of PH3. The study will evaluate the safety and efficacy of a single dose of nedosiran in participants with PH3. The Company anticipates top-line results from the study mid-year.

- **Novo Nominated First Candidate for Development Under RNAi Discovery and Development Agreement.** In January 2021, the Company announced that, at the end of 2020, Novo Nordisk A/S (“Novo”) nominated its first candidate under the existing agreement between the two companies for the discovery and development of novel therapies for the treatment of liver-related cardiometabolic diseases using Dicerna’s proprietary GalXC RNAi technology. Dicerna earned a $2.5 million

milestone associated with nomination of the first development candidate. The Company also received in February 2021 a $25.0 million payment related to the delivery of RNAi molecules for a defined number of targets to Novo in 2020.

- **Completed Enrollment in PHYOX2 Pivotal Trial of Nedosiran for the Treatment of PH.** In January 2021, Dicerna announced enrollment completion of its PHYOX2 pivotal, double-blind, randomized, placebo-controlled clinical trial of its lead investigational therapy, nedosiran, which is in development as a once-monthly treatment for PH1, PH2 and PH3. The Company expects the last patient visit in the PHYOX2 trial to occur in the first half of 2021 and to report top-line results from the study mid-year.

- **Positive Data From Phase 1 Trial of RG6346 for Treatment of Chronic Hepatitis B Virus (HBV) Infection.** Dicerna provided two interim updates of Phase 1 data for RG6346, which is being developed in collaboration with Roche for the treatment of chronic HBV. The most recent data presentation was at The American Association for the Study of Liver Diseases' The Liver Meeting ® Digital Experience™ 2020 in November. Data from the Phase 1 double-blind, placebo-controlled, proof-of-concept trial showed four monthly doses of RG6346 treatment resulted in substantial and durable reductions in hepatitis B surface antigen (HBsAg) levels lasting up to one year after the last dose.

- **U.S. Food and Drug Administration (FDA) Accepted Lilly's IND Application for First GalXC RNAi Candidate Under Companies' Global Research Collaboration and Licensing Agreement.** In November 2020, Dicerna announced that the FDA accepted the IND filed by Eli Lilly and Company (Lilly) for LY3561774, the first clinical-stage candidate to emerge from Dicerna's collaboration with Lilly. Targeting the *ANGPTL3* gene for the treatment of dyslipidemia, the IND milestone achievement triggered a $10.0 million payment to Dicerna.

- **Presented New Nonclinical Research Highlighting Application of RNAi Technology in Extrahepatic Tissues at TIDES Europe 2020.** In November 2020, Dicerna's presentation titled, "Progress on a Pipeline of Extrahepatic RNAi Therapeutics," included nonclinical data demonstrating messenger RNA (mRNA) knockdown activity in multiple extrahepatic tissues, including the central nervous system, adipose, skeletal muscle, adrenal and other tissues in rodents and nonhuman primates using therapeutic nucleic acid modalities that leverage the Company's proprietary GalXC-Plus RNAi technology.

- **Presented Positive Interim Data From PHYOX3 Long-Term, Open-Label Extension Study of Nedosiran for Treatment of PH.** In October 2020, Dicerna presented updated positive interim data from its ongoing PHYOX3 open-label trial of once-monthly nedosiran suggesting that nedosiran may meaningfully reduce hepatic oxalate overproduction, potentially slowing disease progression.

  These data were presented at the American Society of Nephrology's Kidney Week 2020 and demonstrated that 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, 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 as defined in the study protocol at one or more visits.

Anticipated Upcoming 2021 Milestones

- **Nedosiran:**
  - Top-line data from PHYOX2 pivotal trial in PH1 and PH2 patients mid-year 2021
  - Top-line data from PHYOX4 trial in PH3 patients mid-year 2021
  - Initiate PHYOX7 trial of PH1 and PH2 patients with severe renal impairment, including those undergoing dialysis, in the first quarter of 2021
  - Initiate PHYOX8 trial, an open-label study in PH1 and PH2 patients aged 0-5 years with relatively intact renal function, in the second quarter of 2021
  - New Drug Application submission near the end of the third quarter of 2021

- **Collaborative Program:** Lilly IND filing for LY3819469 in the second quarter of 2021

- **RG6346:** Roche to initiate RG6346 in Phase 2 combination trial in the first quarter of 2021

- **Belcesiran:** Initiate Phase 2 trial in the first half of 2021 and present initial Phase 1 trial data mid-year 2021

- **DCR-AUD:** File IND mid-year 2021 and initiate Phase 1 study in healthy volunteers in the third quarter of 2021

Financial Results for Full Year Ended Dec. 31, 2020

- **Cash Position** – As of Dec. 31, 2020, Dicerna had $568.8 million in cash, cash equivalents and held-to-maturity investments, compared to $348.9 million as of Dec. 31, 2019.

- **Revenue** – Dicerna recognized $164.3 million for the full year 2020, compared to $23.9 million in 2019. The increase in revenue on a year-over-year basis was primarily attributable to increased activities and associated costs under the recent collaboration agreement with Roche, as well as under the Alexion and Lilly collaboration agreements.

- **Research and Development (R&D) Expenses** – R&D expenses were $205.4 million for the full year 2020, compared to $109.3 million in 2019. The increase was primarily due to increases in nedosiran and other direct research and development expenses and an increase in employee-related expenses.

- **General and Administrative (G&A) Expenses** – G&A expenses were $72.1 million for the full year 2020, compared $42.8 million in 2019. The increase was primarily due to employee-related expenses as a result of increased headcount necessary to support our growing operations and an increase in professional consulting fees.

- **Net Loss** – Net loss was $112.7 million, or $1.52 per share, for the full year ended Dec. 31, 2020, compared to a net loss of $120.5 million, or $1.76 per share, for the full year 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. Through the year ending Dec. 31, 2021, we forecast receiving over $100.0 million in cash from our collaborations, inclusive of the receipt of $17.5 million in the three months ended December 31, 2020, including payments from anticipated milestone achievements.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's full year 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 9592143 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 9592143. 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-based GalXC technology, yet 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 rare, 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 product candidates and the development thereof, such as the newly announced clinical development candidate DCR-AUD for AUD, including the progress of and anticipated milestones for the Company’s PHYOX4 and PHYOX2 ongoing and planned trials, including those from its PHYOX program as well as other trials of nedosiran, results from future 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, 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, and any potential future collaborations. 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 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 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

CONSOLIDATED BALANCE SHEETS (In thousands)	**DECEMBER 31, 2020**	**DECEMBER 31, 2019**
Cash and cash equivalents	$ 126,023	$ 152,816
Held-to-maturity investments	442,820	196,065
Restricted cash equivalents, current	744	—
Contract receivables	34,713	200,354
Prepaid expenses and other current assets	14,403	6,934
Property and equipment, net	17,546	7,076
Right-of-use operating assets, net	60,843	30,102
Restricted cash equivalents, noncurrent	5,618	3,894
Other noncurrent assets	5,136	168
Total Assets	$ 707,846	$ 597,409
Accounts payable	$ 7,901	$ 6,077
Accrued expenses and other current liabilities	31,500	23,400
Deferred revenue, current	138,537	212,258
Deferred revenue, noncurrent	336,236	182,730
Other noncurrent liabilities	55,918	20,749
Total stockholders' equity	137,754	152,195
Total Liabilities and Stockholders' Equity	$ 707,846	$ 597,409
Common stock outstanding	75,757	71,573

CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Year Ended December 31, 2020**	**Year Ended December 31, 2019**
Revenue	$ 164,307	$ 23,904
Operating expenses:		
Research and development	205,384	109,339
General and administrative	72,131	42,751
Total operating expenses	277,515	152,090
Loss from operations	(113,208)	(128,186)
Other income (expense):		
Interest income (expense), net	5,991	7,534
Other (expense) income	(5,530)	193
Total other income, net	461	7,727
Net loss	$ (112,747)	$ (120,459)
Net loss per share – basic and diluted	$ (1.52)	$ (1.76)
Weighted average common shares outstanding – basic and diluted	74,187	68,428

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