Dicerna™ Announces Fourth Quarter and Full Year 2019 Financial Results and Provides Corporate Update

February 27, 2020

— Company Achieves Multiple Clinical and Corporate Development Milestones in 2019, Advancing Core Pipeline Candidates; Expanding and Progressing Existing Collaborative Programs; and Establishing New, High-Value Collaborations With Roche and Novo Nordisk A/S —

— Anticipated 2020 Milestones Include: First Nedosiran Multi-dose Data From PHYOX™3 Trial; RG6346 Phase 1 Proof-of-Concept Data; Patien Dosing in Phase 1/2 Trial of DCR-A1AT; and Nedosiran PHYOX2 Pivotal Trial Last Patient Out —

—$348.9 Million in Cash, Cash Equivalents and Marketable Securities at Dec. 31, 2019; $375 Million in Upfront Payments Related to Roche and Novo Nordisk Collaborations Received in January 2020 —

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

LEXINGTON, Mass.--(BUSINESS WIRE)-- Dicerna™ Pharmaceuticals, Inc. (Nasdaq: DRNA) (the “Company” or “Dicerna”), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial results for the fourth quarter and full year ended Dec. 31, 2019, and provided a corporate update.

“Dicerna continued its rapid maturation in 2019,” commented Douglas Fambrough, Ph.D., president and chief executive officer of Dicerna. “Within our core clinical pipeline, we initiated pivotal development of nedosiran, our lead product candidate for primary hyperoxaluria types 1, 2 and 3 and initiated patient dosing of DCR-HBVS, now referred to as RG6346, observing multiple patients with greater than or equal to one $\log_{10}$ IU/ml HBsAG reduction in this ongoing, blinded study. We are encouraged to now have representation from both Groups B and C of our Phase 1 study of RG6346 in the extended follow-up phase. We also began clinical development of DCR-A1AT, all while progressing and expanding programs under our existing collaboration agreements. In 2019, we also established new, high-value strategic relationships with Roche and Novo Nordisk that align with our two-part business strategy to develop core programs that are complemented by broad collaborative discovery relationships that have the potential to provide additional revenue and growth opportunities in the coming years.

“2020 will be defined by clinical execution across our core portfolio, establishing the foundation for anticipated commercialization of nedosiran, and expansion and advancement of our early GalXC™ RNAi pipeline both for ourselves and our collaborative partners,”Dr. Fambrough continued. “Our business development activities last year meaningfully augment our cash position, providing us with the financial resources to achieve our clinical, collaborative and business goals into 2023 and support our evolution to a fully integrated, commercial-stage biopharmaceutical company.”

Recent Events

- **Closing of Collaboration and Licensing Agreement With Novo Nordisk A/S (Novo Nordisk) and Receipt of $175 Million Upfront Payment and Proceeds From $50 Million Equity Investment in Dicerna.** On Jan. 21, 2020, Dicerna received a $175 million upfront payment in connection with the closing and successful delivery of a program start-up package related to the discovery and development agreement between Novo Nordisk and Dicerna announced in November 2019. In December 2019, Novo Nordisk completed its $50 million equity investment in Dicerna in connection with the agreement. The collaboration includes the discovery and development of novel therapies for the treatment of liver-related cardiometabolic diseases using Dicerna’s proprietary GalXC RNAi platform technology. The collaboration is expected to explore more than 30 liver cell targets and may deliver multiple clinical candidates in areas including chronic liver disease, non-alcoholic steatohepatitis (NASH), type 2 diabetes, obesity and rare diseases. The agreement also provides Dicerna and Novo Nordisk the option to co-develop and co-commercialize two product candidates each discovered under the collaboration. Dicerna is eligible to receive development, regulatory and commercialization milestone payments, plus tiered royalties on potential product sales.

- **Closing of Worldwide Collaboration and Licensing Agreement With Roche and Receipt of $200 Million Upfront Payment.** On Jan. 7, 2020, Dicerna received a $200 million upfront payment related to the successful closing of the research collaboration and licensing agreement between Roche and Dicerna announced in October 2019. The collaboration includes the development of novel therapies for the treatment of chronic hepatitis B virus (HBV) infection using Dicerna’s proprietary GalXC RNAi platform technology. The collaboration will focus on worldwide development and commercialization of RG6346, an investigational therapy in Phase 1 clinical development, and the discovery and development of therapies targeting human genes associated with HBV infection, or additional targets within the HBV genome, using the technology platforms of both companies. Under the collaboration, Dicerna is eligible to receive up to $1.47 billion in potential milestone payments related to RG6346 as well as royalties on product sales. The agreement also provides Dicerna with the option to co-fund development of products under the agreement and, if exercised, receive enhanced royalty rates on the net sales of products in the U.S. and an option to co-promote products containing RG6346 in the U.S.

- **Orphan Drug Designation for DCR-A1AT in the European Union.** In December 2019, the European Commission

granted orphan drug designation to Dicerna's DCR-A1AT for the treatment of congenital alpha-1 antitrypsin (A1AT) deficiency based on a positive opinion from the Committee for Orphan Medicinal Products of the European Medicines Agency. Dicerna is conducting a clinical trial program investigating DCR-A1AT for the treatment of A1AT deficiency-associated liver disease.

- **Alexion Pharmaceuticals, Inc. (Alexion) Exercised Options for Two Additional Targets.** In December 2019, Alexion exercised its options for exclusive rights to two additional targets within the complement pathway for the discovery and development of GalXC RNAi molecules, expanding the companies' existing research collaboration and license agreement to now encompass four targets. In connection with the option exercise, Alexion paid Dicerna a total of $20 million, or $10 million in option exercise fees per additional new target.

- **Appointment of Stephen Doberstein, Ph.D., to Board of Directors.** In February 2020, Dicerna announced the appointment of Stephen Doberstein, Ph.D., Chief Scientific Fellow at Nektar Therapeutics, Inc., to Dicerna's board of directors as an independent director.

- **Completed Sale of Common Stock.** In February 2020, Dicerna issued and sold an aggregate of approximately $40.0 million of shares of common stock to a single institutional investor through the Company's at-the-market facility under an existing shelf registration statement. In this transaction, the Company sold an aggregate of 2,077,500 shares of common stock at a price of $19.25 per share.

Expected Upcoming Milestones and Events

- **Nedosiran**: First multi-dose data from PHYOX ™3 open-label clinical trial – OxalEurope International Congress, March 31, 2020
- **Nedosiran**: PHYOX2 pivotal clinical trial enrollment completion – second quarter of 2020
- **R&D Day and Corporate Update** – third quarter of 2020
  - **RG6346**: Present Phase 1 proof-of-concept data from all existing cohorts
  - **Nedosiran**: Additional multi-dose data from PHYOX3 open-label clinical trial
  - **GalXC**: Present data for extending GalXC technology to additional tissues
- **DCR-A1AT**: First patient dosing in Phase 1/2 trial – second half of 2020
- **Collaborative Program**: Investigational New Drug or Clinical Trial Authorization filing for LY3561774 – late 2020
- **Nedosiran**: PHYOX2 last patient out – year-end 2020

Financial Condition and Operating Results for the Fourth Quarter of 2019

- **Cash Position** – As of Dec. 31, 2019, Dicerna had $348.9 million in cash, cash equivalents, and held-to-maturity investments, compared to $302.6 million as of December 31, 2018. Additionally, the Company had $3.9 million and $0.7 million of restricted cash equivalents as of Dec. 31, 2019 and Dec. 31, 2018, respectively, reflecting collateral securing the Company's lease obligations.
- **Revenue** – Dicerna recognized $7.1 million of revenue associated with its collaboration agreements during the quarter ended Dec. 31, 2019, compared to $1.5 million for the same period in 2018.
- **Research and Development (R&D) Expenses** – R&D expenses were $34.8 million for the quarter ended Dec. 31, 2019, compared to $13.8 million for the same period in 2018. The increase was primarily due to increased manufacturing costs, clinical study costs, and employee-related expenses due to an increase in headcount necessary to support our growth.
- **General and Administrative (G&A) Expenses** – G&A expenses were $13.6 million for the quarter ended Dec. 31, 2019, compared to $7.2 million for the same period in 2018. The increase was primarily due to employee-related expenses as a result of increased headcount necessary to support our growth, as well as an increase in professional fees and consulting expenses.
- **Net Loss** – Net loss was $39.7 million, or $0.58 per share, for the quarter ended Dec. 31, 2019, compared to $18.6 million, or $0.29 per share, for the same period in 2018.

Guidance

Dicerna believes that its cash, cash-equivalents and held-to-maturity investments will be sufficient to fund the execution of its current clinical and operating plan into 2023, which includes our expectations to advance nedosiran through pivotal development, regulatory filing and potential commercial launch; completing proof-of-concept studies of RG6346 in participants with HBV infection; advancing the Company's DCR-A1AT program through the initial Phase 1/2 clinical study; and initiating and conducting research and development programs with our collaborative partners. This estimate assumes no new funding from additional 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 significantly for the foreseeable future, primarily as the Company continues clinical manufacturing activities, advances preclinical toxicology studies, continues clinical activities associated with its lead product candidates, prepares for commercialization of nedosiran and increases or continues activities under the agreements with Novo Nordisk, Roche, Eli Lilly and Company, Alexion and Boehringer Ingelheim International GmbH.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's fourth quarter 2019 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 3259673 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 (404) 537-3406 and refer to conference ID 3259673. The webcast will also be archived on Dicerna’s website.

About Dicerna's GalXC™ RNAi Technology Platform

Dicerna’s proprietary ribonucleic acid interference (RNAi) technology platform, called GalXC™, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver and other body systems. Liver-targeted GalXC-based compounds enable subcutaneous delivery of RNAi therapies that are designed to specifically bind to receptors on liver cells, leading to internalization and access to the RNAi machinery within the cells. The GalXC approach seeks to optimize the activity of the RNAi pathway so that it operates in the most specific and potent fashion. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, including both liver and non-liver indications.

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 selectively silence 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 reducing the level of disease-causing proteins in the hepatocytes of the liver, Dicerna’s GalXC has the potential to safely target conditions that are difficult to treat with other modalities. Continually innovating, Dicerna is also exploring new applications of 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. and Boehringer Ingelheim International GmbH. Between Dicerna and our collaborative partners, we currently have more than 20 active discovery, preclinical or clinical programs focused on rare, cardiovascular, cardiometabolic, viral-infectious, 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 heath. 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) the therapeutic and commercial potential of nedosiran, RG6346, DCR-A1AT and the GalXC™ platform; (ii) research and development plans and timelines related to nedosiran, RG6346, DCR-A1AT and GalXC, including continued alignment on the regulatory pathway to approval of nedosiran; (iii) the potential for Dicerna to continue to add programs and expand collaborative work with our liver-targeted GalXC technology and to extend the reach of our GalXC technology to additional tissues in our internal discovery research and in our collaborative programs; (iv) the potential of Dicerna’s technology and drug candidates in the Company’s research and development pipeline; and (v) Dicerna’s financial position, expectations about current or future collaboration funding, expenses and cash runway. The process by which investigational therapies, some of which are early-stage, such as nedosiran, RG6346, DCR-A1AT, our collaborative research and development programs and an early-stage platform such as GalXC 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-Q and 10-K 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 unpredictability of the duration and results of the regulatory review of Investigational New Drug Applications (NDAs) and Clinical Trial Applications that are necessary to continue to advance and progress the Company’s clinical programs and the regulatory review of NDAs; 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)	**December 31, 2019**	**December 31, 2018**
Cash and cash equivalents	$ 152,816	$ 54,239
Held-to-maturity investments	196,065	248,387
Contract receivables	200,354	100,000
Prepaid expenses and other current assets	6,934	2,888
Property and equipment, net	7,076	2,718
Right-of-use operating assets, net	30,102	—
Restricted cash equivalents	3,894	744

Other noncurrent assets	168	65
Total Assets	$ 597,409	$ 409,041
Accounts payable	$ 6,077	$ 5,013
Accrued expenses and other current liabilities	20,042	9,649
Lease liability, current	3,358	—
Deferred revenue, current	212,258	68,893
Litigation settlement payable	—	10,500
Lease liability, noncurrent	20,141	—
Deferred revenue, noncurrent	182,730	114,293
Other noncurrent liabilities	608	—
Total stockholders' equity	152,195	200,693
Total Liabilities and Stockholders' Equity	$ 597,409	$ 409,041
Common stock outstanding	71,573	68,211

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Three Months Ended December 31, 2019**	**Three Months Ended December 31, 2018**
Revenue	$ 7,080	1,541
Operating expenses:		
Research and development	34,818	13,784
General and administrative	13,625	7,235
Litigation expense	—	10
Total operating expenses	48,443	21,029
Loss from operations	(41,363)	(19,488)
Other income (expense):		
Interest income	1,503	1,082
Interest expense	(3)	(204)
Other income (expense)	193	—
Total other income, net	1,693	878
Net loss	$ (39,670)	(18,610)
Net loss per share – basic and diluted	$ (0.58)	(0.29)
Weighted-average common shares outstanding – basic and diluted	68,763	63,268

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (In thousands, except per share data)	**Year Ended December 31, 2019**	**Year Ended December 31, 2018**
Revenue	$ 23,904	$ 6,176
Operating expenses:		
Research and development	109,339	45,711
General and administrative	42,751	21,685
Litigation expense	—	29,132
Total operating expenses	152,090	96,528
Loss from operations	(128,186)	(90,352)
Other income (expense):		
Interest income	7,537	2,102
Interest expense	(3)	(603)
Other income (expense)	193	—
Total other income, net	7,727	1,499
Net loss	$ (120,459)	$ (88,853)
Net loss per share – basic and diluted	$ (1.76)	$ (1.60)
Weighted-average common shares outstanding – basic and diluted	68,428	55,616

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