



Dicerna™ Reports Fourth Quarter and Year Ended December 31, 2018 Financial Results and Provides Corporate Update

March 11, 2019

— 2018 was a Milestone Year for Dicerna Across a Variety of Fronts, Including Data Readouts for Primary Hyperoxaluria, Business Development Collaborations and a Successful Follow-On Offering —

—Company's Robust Cash Position Provides Funds Beyond 2020 to Fuel Pipeline Advancement and Growth —

— Management to Host Conference Call Today at 3:30 p.m. ET —

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 11, 2019-- [Dicerna™ Pharmaceuticals, Inc.](#) (Nasdaq:DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operating results for the fourth quarter and year ended December 31, 2018.

"2018 was a year of tremendous achievement for Dicerna. During the year, we met each of our key strategic objectives, as we obtained strong proof-of-concept (POC) data for our lead program, DCR-PHXC for primary hyperoxaluria (PH), for which we plan to initiate a pivotal trial in the near-term. In addition, we are continuing to advance our pipeline, which includes our investigational therapy, DCR-HBVS, for the treatment of people with chronic hepatitis B virus (HBV) infection and an additional candidate for an undisclosed rare disease," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Further, we signed two major business development deals, executed a successful \$115 million follow-on offering and solidified our intellectual property position."

"Our active business development and financing activities have put us in a strong financial position ending 2018 with \$302.6 million of cash, cash equivalents and held-to-maturity investments in addition to \$94.5 million of net proceeds received in early 2019 from our collaboration agreements. Our strong cash position enables us to advance clinical trials for PH and chronic HBV infection, along with additional pipeline assets," said Jack Green, chief financial officer of Dicerna. "We expect to be sufficiently funded beyond 2020 and are now well on our way to building a fully integrated company comprised of both proprietary and partnered programs."

Recent Achievements

Development Programs

- On January 28, 2019, Dicerna announced the dosing of the first healthy volunteer (HV) in the multi-dose, double-blind, randomized, placebo-controlled Phase 1 DCR-HBVS clinical trial, studying the Company's investigational GalXC™-based therapy for the treatment of chronic HBV infection in adults. Dosing of the first patient with HBV in the second portion of the study is expected to occur early in the second quarter of 2019. The Company anticipates human POC data to be available in the fourth quarter of 2019.
- On October 25, 2018, clinical investigators presented clinical POC data for DCR-PHXC candidate in the ongoing PHYOX™ Phase 1 clinical trial, at the American Society of Nephrology Kidney Week 2018 annual meeting. Initial findings as of a data analysis on October 1, 2018 were associated with normalization or near-normalization of urinary oxalate levels in a majority of adults with primary hyperoxaluria types 1 and 2 (PH1 and PH2). Investigators reported that a single 3.0 mg/kg dose of DCR-PHXC brought urinary oxalate levels into the normal range (defined as 24-hour excretion <0.46 mmol) at one or more post-dose time points in three out of four participants, including a mean maximal reduction in 24-hour urinary oxalate of 65%. Investigators also reported that a single 1.5 mg/kg dose led to near-normalization (defined as 24-hour excretion <0.600 and ≥0.460 mmol) in three out of four PH1 participants and led to a mean maximal reduction in urinary oxalate of 50% in the five patients dosed at that level, including one PH2 patient. Preliminary results also showed that DCR-PHXC was safe and well-tolerated in both HVs and participants with PH. At this point, all patients have completed dosing. The Company remains on track to initiate PHYOX2, the multi-dose, double-blind, placebo-controlled pivotal trial of DCR-PHXC, during the first quarter of 2019.
- Dicerna has continued to advance internal development of its wholly-owned undisclosed program for a rare disease involving the liver, currently in Investigational New Drug (IND)-enabling studies. Submissions of regulatory filings are planned in the second quarter of 2019.

Collaborations

- On January 4, 2019, Dicerna announced Boehringer Ingelheim GmbH's (BI) decision to exercise its option for a second hepatic disease target under the existing collaboration and license agreement, triggering a \$5 million payment to Dicerna in Q1 2019.
- On December 19, 2018, the Company announced the closing of a collaboration and license agreement with Eli Lilly and Company (Lilly) for the discovery, development and commercialization of potential new medicines in the areas of cardiometabolic disease, neurodegeneration and pain. The collaboration, which contemplates more than 10 targets, yielded \$100 million in upfront cash in January 2019 and a \$100 million equity investment at a premium in 2018, and provides

Dicerna with a unique opportunity to leverage the GalXC platform to key tissues beyond the liver.

- On October 24, 2018, Dicerna announced the closing of a collaborative research and license agreement with Alexion Pharmaceuticals, Inc. (Alexion). The agreement generated \$37 million in upfront cash, including a \$15 million equity investment at a premium. The agreement provides for the joint discovery and development of subcutaneously delivered GalXC RNAi molecules directed to two complement pathway targets for the treatment of complement-mediated diseases. In addition, Alexion will have the right to exercise options, for additional payments, for two additional GalXC RNAi molecules directed to complement pathway targets.

Business Highlights

- On January 24, 2019, Dicerna strengthened its board of directors (the Board):
 - Elevated J. Kevin Buchi, former chief executive officer of Cephalon, Inc. and TetraLogic Pharmaceuticals Corp., to chairman of the Board.
 - Appointed industry experts Marc Kozin, professional board member, and Anna Protopapas, president and chief executive officer of Mersana Therapeutics, Inc.
- On September 6, 2018, the Company announced pricing of an equity financing which yielded gross proceeds of \$115 million providing significant additional resources to progress the Company's clinical and operational objectives.

Upcoming Events

- On track to initiate a multi-dose, double-blind, randomized, placebo-controlled pivotal trial of DCR-PHXC named PHYOX2 in the first quarter of 2019.
 - On track to initiate PHYOX3, a long-term, multi-dose, open-label, registration roll-over extension initially for the Company's Phase 1 study in the second quarter of 2019, enabling continuous readouts of multi-dose data.
 - Additional supportive trials are planned, including an open-label study in patients with PH type 3 and a single dose trial in adults with end-stage renal disease (ESRD), aimed at determining pharmacokinetics and an open-label pediatric study in children ages 2-5 years.
- On track to dose patients with chronic HBV of the Phase 1 clinical trial of DCR-HBVS in the second quarter of 2019.
 - Human POC data is anticipated to be available in the fourth quarter of 2019.
- On track to submit a Clinical Trial Application filing for the Company's undisclosed rare disease program in the second quarter of 2019.

Financial and Operating Results

- **Cash Position** – As of December 31, 2018, Dicerna had \$302.6 million in cash, cash equivalents and held-to-maturity investments, and additionally received \$94.5 million of net proceeds in early 2019 from the collaboration agreements, which enables Dicerna to advance clinical trials for PH, HBV infection, and additional pipeline assets, as compared to \$113.7 million in cash, cash equivalents and held-to-maturity investments, respectively, as of December 31, 2017.
- **Revenue** – During the year ended December 31, 2018, revenue from collaborative arrangements increased \$5.1 million, as compared to 2017, due to recognition of a full year of revenue under the BI agreement and initial revenue recognized under the recent collaboration with Alexion.
- **Research and Development (R&D) Expenses** – R&D expenses were \$45.7 million and \$35.9 million for the years ended December 31, 2018 and 2017, respectively, an increase of \$9.8 million. This increase was primarily due to a \$6.3 million increase in clinical study costs due to increased activities associated with the DCR-PHXC and DCR-HBVS programs as well as an increase of \$3.0 million in employee-related expenses associated with increased headcount to support growth.

Dicerna expects overall R&D expenses to increase in 2019, as compared to 2018, and for the foreseeable future, primarily as the Company completes clinical manufacturing activities, advances preclinical toxicology studies, continues clinical activities associated with its lead product candidates and initiates activities under the recently signed Lilly and Alexion agreements.

- **General and Administrative (G&A) Expenses** – G&A expenses were \$21.7 million and \$16.8 million for the years ended December 31, 2018 and 2017, respectively. The increase of approximately \$5.0 million is primarily due to increases in consulting costs of \$1.9 million, board of directors' compensation of \$1.0 million and salary and benefits expenses of \$0.8 million.

Dicerna expects G&A expenses to increase in 2019, as compared to 2018, largely due to investment in staffing, preparation of the Company's new headquarters and market readiness activities.

- **Litigation Expense** – Litigation expenses increased primarily due to \$24.7 million of Alnylam Pharmaceuticals, Inc. settlement expenses during the year ended December 31, 2018. The final payment under the settlement was made in January 2019.

- **Net Loss Attributable to Common Stockholders** – Net loss attributable to common stockholders was \$88.9 million and \$80.3 million for the years ended December 31, 2018 and 2017, respectively. The overall increase in net loss attributable to common stockholders was driven by the \$28.7 million increase in net loss due to the factors discussed above, offset by \$20.1 million in dividends and deemed dividends on the Redeemable Convertible Preferred stock recorded in 2017.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's fourth quarter and year ended December 31, 2018 financial results and provide a general business update. The conference call can be accessed by dialing (855) 453-3834 or (484) 756-4306 (international) and referencing conference ID 3386549 prior to the start of the call. The call will also be webcast via the internet and will be available under the "Investors & Media" section of the Dicerna website, www.dicerna.com. A replay of the call will be available for seven days. To access the replay, please dial (855) 859-2056 or (404) 537-3406 and refer to conference ID 3386549. The webcast will also be archived on Dicerna's website.

About Dicerna™ Pharmaceuticals, Inc.

Dicerna™ Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative, subcutaneously delivered RNAi-based therapeutics for the treatment of diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases and viral infectious diseases. Dicerna is leveraging its proprietary GalXC™ RNAi technology platform to build a broad pipeline in these core therapeutic areas, focusing on target genes where connections between target gene and diseases are well understood and documented. Dicerna intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. Dicerna has strategic collaborations with Eli Lilly and Company, Alexion Pharmaceuticals, Inc. and Boehringer Ingelheim International GmbH. For more information, please visit www.dicerna.com.

About GalXC™ RNAi Technology Platform

GalXC™ is a proprietary technology platform invented by Dicerna to discover and develop RNAi-based therapies designed to silence disease-driving genes in the liver. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases and viral infectious diseases. Using GalXC, Dicerna scientists attach *N*-acetylgalactosamine sugars directly to the extended region of the Company's proprietary RNAi molecules, yielding multiple proprietary conjugate delivery configurations. Many of the conjugates produced via GalXC incorporate a folded motif known as a tetraloop in the extended region. The tetraloop configuration, which is unique to Dicerna's GalXC compounds, allows flexible and efficient conjugation to the targeting ligands, and stabilizes the RNAi duplex which the Company believes will enable subcutaneous delivery of its RNAi therapies to hepatocytes in the liver, where they are designed to specifically bind to receptors on target cells, potentially leading to internalization and access to the RNAi machinery within the cells. The technology may offer several distinct benefits, as suggested by strong preclinical data. The benefits seen in preclinical studies include: potency that is on par with or better than comparable platforms; highly specific binding to gene targets; long duration of action; and an infrequent subcutaneous dosing regimen.

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 DCR-PHXC, DCR-HBVS and the GalXC platform; (ii) research and development plans and timelines related to DCR-PHXC, GalXC, including DCR-HBVS; and (iii) the potential of Dicerna's technology and drug candidates in the Company's research and development pipeline. The process by which an early stage investigational therapy such as DCR-HBVS 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 Form 10-Q filing 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; 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 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 CTAs that are necessary to continue to advance and progress the Company's clinical programs and the regulatory review of NDAs; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain and costs of obtaining intellectual property rights; and possible safety or efficacy concerns that could emerge as new data are generated in R&D, 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.

Dicerna™, GalXC™, and PHYOX™ are trademarks of Dicerna Pharmaceuticals, Inc.

DICERNA PHARMACEUTICALS, INC. SELECTED FINANCIAL INFORMATION (UNAUDITED)

Condensed Consolidated Balance Sheets (In thousands)	December 31,	
	2018	December 31, 2017
Cash and cash equivalents	\$ 54,239	\$ 68,789
Held-to-maturity investments	248,387	44,889
Contract receivables	100,000	—
Other current assets	2,888	4,998

Property and equipment, net	2,718	1,512
Restricted cash equivalents	744	744
Other noncurrent assets	65	70
Total Assets	\$ 409,041	\$ 121,002
Accounts payable	\$ 5,013	\$ 4,920
Accrued expenses and other current liabilities	9,649	5,726
Litigation settlement payable	10,500	—
Current portion of deferred revenue	68,893	6,180
Deferred revenue, net of current portion	114,293	3,090
Total stockholders' equity	200,693	101,086
Total Liabilities and Stockholders' Equity	\$ 409,041	\$ 121,002
Common stock outstanding	68,211	51,645

DICERNA PHARMACEUTICALS, INC.
SELECTED FINANCIAL DATA (UNAUDITED)

Condensed Consolidated Statements of Operations (In thousands, except per share data)	Three Months Ended December 31, 2018	Three Months Ended December 31, 2017
Revenue from collaborative arrangements	\$ 1,541	\$ 1,030
Operating expenses:		
Research and development	13,784	9,550
General and administrative	7,235	4,513
Litigation expense	10	2,887
Total operating expenses	21,029	16,950
Loss from operations	(19,488) (15,920
Other income (expense):		
Interest income	1,082	179
Interest expense	(204) —
Total other income, net	878	179
Net loss	(18,610) (15,741
Dividends on redeemable convertible preferred stock	—	(3,378
Deemed dividend on conversion of redeemable convertible preferred stock	—	(3,837
Net loss attributable to common stockholders	\$ (18,610) \$ (22,956
Net loss per share attributable to common stockholders – basic and diluted	\$ (0.29) \$ (0.91
Weighted-average common shares outstanding – basic and diluted	63,268	25,205

DICERNA PHARMACEUTICALS, INC.
SELECTED FINANCIAL DATA (UNAUDITED)

Condensed Consolidated Statements of Operations (In thousands, except per share data)	Year Ended December 31, 2018	Year Ended December 31, 2017
Revenue from collaborative arrangements	\$ 6,176	\$ 1,030
Operating expenses:		
Research and development	45,711	35,888
General and administrative	21,685	16,838
Litigation expense	29,132	9,043
Total operating expenses	96,528	61,769
Loss from operations	(90,352) (60,739
Other income (expense):		
Interest income	2,102	539
Interest expense	(603) —
Total other income, net	1,499	539
Net loss	(88,853) (60,200
Dividends on redeemable convertible preferred stock	—	(10,111
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	(6,144
Deemed dividend on conversion of redeemable convertible preferred stock	—	(3,837
Net loss attributable to common stockholders	\$ (88,853) \$ (80,292

Net loss per share attributable to common stockholders – basic and diluted	\$ (1.60) \$ (3.66)
Weighted-average common shares outstanding – basic and diluted	55,616	21,917	

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Source: Dicerna Pharmaceuticals, Inc.

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