March 30, 2017

# Dicerna Reports Fourth Quarter and Full Year 2016 Financial and Operational Results

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Dicerna Pharmaceuticals, Inc. (NASDAQ: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operational results for the fourth quarter and full year ended December 31, 2016.

"2016 was an important year for Dicerna as we completed the transition to our GalXC™ RNAi technology platform and focused our efforts squarely on driving the development of our GalXC pipeline programs," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "The ability of GalXC to enable development of subcutaneously delivered RNAi therapeutics to silence any disease-causing gene in the liver provides substantial opportunities for growth. Our $70.0 million convertible preferred financing, announced today, to be led by Bain Capital Life Sciences and a syndicate of current and new investors, further supports the broad applicability of our GalXC RNAi technology and will be key to progressing our pipeline and strategic plan over the next two years.

"Our two-pronged strategy includes internal development of product candidates for rare diseases that are genetically and molecularly defined, have high unmet medical need, and offer efficient development paths. Our lead product candidate, DCR-PHXC, for primary hyperoxaluria type 1, as well as our second development program aimed at an undisclosed rare disease, fall into this category. Additionally, we are aggressively pursuing key partnerships for programs that address complex diseases with multiple gene dysfunctions and larger patient populations, including DCR-PCSK9 for hypercholesterolemia and DCR-HBV for hepatitis B virus."

**GalXC™ Program Update**

- During 2016, Dicerna continued to optimize its GalXC technology platform and expand its preclinical pipeline of product candidates that are long-acting and highly specific for targets in the liver within its core therapeutic areas of rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases.
  - Dicerna has prioritized four therapeutic programs where it believes the probability of success is favorable; these include DCR-PHXC, an undisclosed rare disease program, DCR-PCSK9, and DCR-HBV. Dicerna plans to file its first Investigational New Drug (IND) application and/or Clinical Trial Application (CTA) for its GalXC product candidates at the end of 2017 followed by additional INDs in 2018 and 2019.
  - The Company has qualified a significant number of disease-associated genes in clinical indications where it believes an RNAi-based inhibitor may provide substantial benefit to patients, providing expansive therapeutic opportunities, and has developed hits and/or optimized GalXC conjugate inhibitors against almost 40 targets.
- Primary Hyperoxaluria Type 1 (PH1): During 2016, Dicerna announced the launch of DCR-PHXC, its first subcutaneously delivered GalXC clinical candidate for the treatment of patients with PH1. PH1 is a rare inborn error of metabolism in which the liver produces excessive levels of oxalate, which in turn causes severe damage to the kidneys and to other tissues in the body. PH1 is often fatal in the absence of a combined liver-kidney transplant.
  - DCR-PHXC is in preclinical development and has advanced into IND-enabling studies. Dicerna intends to file an IND submission and/or CTA for DCR-PHXC in late 2017 and commence Phase 1 clinical trials in the first quarter of 2018.
  - To facilitate development of DCR-PHXC, Dicerna continues to advance its Primary HYperoxaluria Observational Study (PHYOS), an international, multicenter, observational study in patients with a genetically confirmed diagnosis of PH1. PHYOS is collecting data on key biochemical parameters implicated in the pathogenesis of PH1. Dicerna hopes to use the data to better understand the baseline PH1 disease state, which will help guide long-term drug development plans.
- Undisclosed Rare Disease Involving the Liver: During the fourth quarter of 2016, Dicerna launched development of a GalXC-based therapeutic with an optimized lead candidate that targets a liver-expressed gene involved in a serious rare disease. Dicerna has selected this target gene and disease based on criteria that include having a strong therapeutic hypothesis, a readily-identifiable patient population, the availability of a potentially predictive biomarker, and high unmet medical need. Dicerna plans to file an IND for this program in the second quarter of 2018.
- Cardiovascular Disease: Based on the Company's candidate development work during the fourth quarter of 2016, Dicerna is positioned to advance DCR-PCSK9, which targets the PCSK9 gene and is indicated for the treatment of

statin-refractory patients with hypercholesterolemia, into formal preclinical development. PCSK9 is a validated target for hypercholesterolemia, and United States Food and Drug Administration-approved therapies targeting PCSK9 using monoclonal antibody (MAb) technology are currently on the market. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform has the potential to produce a PCSK9-targeted therapy with more attractive commercial properties than existing MAb therapies, based on comparatively smaller subcutaneous injection volumes and less frequent dosing, while providing equal or superior control of serum cholesterol.

- Chronic Hepatitis B Virus (HBV): Based on the Company's candidate development work during the fourth quarter of 2016, Dicerna is positioned to advance DCR-HBV, which targets the HBV directly, into formal preclinical development. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg). Based on preclinical studies, Dicerna believes that its GalXC RNAi platform has the potential to produce an experimental HBV-targeted therapy that eliminates HBsAg expression in HBV patients and can be delivered in a commercially attractive subcutaneous dosing paradigm.
- Additional GalXC Programs**:** Dicerna has the capacity to launch up to three programs annually, and intends to advance five programs into the clinic by the end of 2019.

**Financial Condition and Operating Results**

- **Cash Position** - As of December 31, 2016, Dicerna had $45.9 million in cash and cash equivalents and held-to-maturity investments as compared to $94.6 million in cash and cash equivalents and held-to-maturity investments as of December 31, 2015. In addition, the Company had $1.1 million of restricted cash equivalents as of December 31, 2016, which reflects collateral securing the Company's operating lease obligation.
- **Research and development (R&D) Expenses** - R&D expenses for the fourth quarter were $9.3 million, compared to $11.3 million for the same period in 2015. The decrease was due primarily to lower spending on discovery and early development and to cost reductions associated with the wind down of Dicerna's DCR-PH1 and DCR-MYC clinical development programs, which the Company announced during the third quarter of 2016. R&D expenses for full year 2016 were $41.7 million compared to $44.0 million for full year 2015. The decrease was due primarily to lower spending in delivery and early development for the Company's lipid nanoparticle programs as these programs have advanced year-over-year into manufacturing and clinical testing before being discontinued, partially offset by additional pre-clinical studies for Dicerna's new GalXC platform as well as by increased employee- and facility-related expenses.
- **General and administrative (G&A) Expenses** - G&A expenses for the fourth quarter were $4.9 million, compared to $4.4 million for the same period in 2015. The increase was primarily attributable to higher legal costs, partially offset by lower payroll-related expenses. G&A expenses for full year 2016 were $18.3 million compared to $19.2 million for full year 2015. The decrease was primarily due to a decrease in employee-related costs partially offset by an increase in professional fees related to legal costs.
- **Net Loss** - Net loss for the fourth quarter was $14.0 million compared to a net loss of $15.6 million for the same period in 2015. Net loss for full year 2016 was $59.5 million, compared to $62.8 million for full year 2015.

For more detailed information and analysis, see Dicerna's Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the Securities and Exchange Commission (SEC) on March 30, 2017.

**Subsequent Event**

- On March 30, 2017, Dicerna announced the signing of a stock purchase agreement with a syndicate of seven current and new investors, led by Bain Capital Life Sciences, for the sale of redeemable convertible preferred stock ("Preferred Stock") for gross proceeds of $70.0 million. The transaction is expected to close on or before April 11, 2017, subject to the satisfaction of customary closing conditions. The Preferred Stock will be convertible into common shares at a conversion price of $3.19 per share. The Company can require conversion if the price of its common stock exceeds $6.38 per share for 45 of 60 days after the achievement of specified partnering and development milestones. Holders of the Preferred Stock will be entitled to a 12% cumulative annual dividend, payable in stock, which can be reduced to 4% upon the achievement of the same milestones. A Form 8-K has been filed with the Securities and Exchange Commission in connection with this transaction.

**Guidance**

Assuming successful closing of the stock purchase agreement, the Company anticipates that it will have sufficient cash to fund the execution of the Company's current operating plan into 2019, including focusing its resources on advancing its first three development programs through proof of concept studies and a fourth program into formal preclinical development. This estimate assumes no additional funding from new partnership agreements or from additional financing events.

**Conference Call**

Management will host a conference call at 4:30 p.m. ET today to review the Company's fourth quarter and full year 2016 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 70365614 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 beginning at 7:30 p.m. ET on March 30, 2017. To access the replay, please dial (855) 859-2056 or (404) 537-3406, and refer to conference ID 70365614. The webcast will also be archived on the Company's website.

**About Dicerna Pharmaceuticals, Inc.**

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative RNAi-based therapeutics for diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. The Company 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. The Company intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. 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 next-generation 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, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. Using GalXC, Dicerna scientists attach N-acetylgalactosamine (GalNAc) sugars directly to the extended region of our proprietary Dicer substrate short-interfering RNA 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 we believe will enable subcutaneous delivery of Dicerna's 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. These benefits 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, including, for example, the closing of our Preferred Stock financing, our expected timeline and plans for development, potential collaborations, and potential therapeutic benefits. 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. Applicable risks and uncertainties include risks that the offering may be delayed or may not occur due to market or other conditions and the satisfaction of customary closing conditions related to the offering, risks relating to our clinical and preclinical research and other risks identified under the heading "Risk Factors" included in our most recent Form 10-K filing and in other future filings with the SEC. 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 Pharmaceuticals, Inc.**
**Consolidated Balance Sheet Information**
**(In thousands)**

| | December 31, 2016 | December 31, 2015 |
|---|---|---|
| Cash and cash equivalents | $ 20,865 | $ 56,058 |
| Held-to-maturity investments | $ 25,009 | $ 38,551 |
| Total assets | $ 51,252 | $ 100,023 |
| Total liabilities | $ 10,044 | $ 9,001 |
| Total stockholders' equity | $ 41,208 | $ 91,022 |

**Dicerna Pharmaceuticals, Inc.**
**Consolidated Statements of Operations Information**
**(In thousands, except share and per share data)**

| | For the Three Months Ended December 31, | | For the Year Ended December 31, | |
|---|---|---|---|---|
| | **2016** | **2015** | **2016** | **2015** |
| Revenue | $ 133 | $ - | $ 295 | $ 184 |
| Operating expenses: | | | | |
| Research and development | 9,337 | 11,263 | 41,694 | 43,971 |
| General and administrative | 4,871 | 4,418 | 18,349 | 19,240 |
| Total operating expenses | 14,208 | 15,681 | 60,043 | 63,211 |
| Loss from operations | (14,075) | (15,681) | (59,748) | (63,027) |
| Interest income | 53 | 46 | 235 | 188 |
| Net loss | $ (14,022) | $ (15,635) | $ (59,513) | $ (62,839) |
| Net loss per share - basic and diluted | $ (0.68) | $ (0.76) | $ (2.87) | $ (3.09) |
| Weighted average shares outstanding - basic and diluted | 20,753,001 | 20,592,840 | 20,719,761 | 20,320,628 |

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