



Dicerna Reports Third Quarter 2018 Financial and Operating Results and Provides Corporate Update

November 5, 2018

Demonstrated Clinical POC for DCR-PHXC in Primary Hyperoxaluria and On Track to Initiate Registration Trial in Q1 2019

Entered into Strategic Collaborations with Lilly and Alexion to Discover and Develop RNAi Therapies Using Dicerna's GalXC™ Technology Platform

Closed \$115.0 Million Financing Strengthening Balance Sheet

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 5, 2018-- [Dicerna Pharmaceuticals, Inc.](#) (NASDAQ:DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today reported financial and operating results for the third quarter ended September 30, 2018.

"The third quarter and recent weeks have marked a key turning point for Dicerna, with the realization of a number of highly significant clinical, pre-clinical, and operational milestones which, together, put us in a strong position to create substantial value as we execute our GalXC™ RNAi therapeutics development strategy," said Douglas M. Fambrough, Ph.D., president and chief executive officer of Dicerna. "Chief among these are attainment of clinical proof-of-concept (POC) for DCR-PHXC, our lead therapeutic candidate in development for primary hyperoxaluria, our recently announced strategic collaboration and licensing agreements with Lilly and Alexion, and the completion of a \$115.0 million follow-on offering. These achievements reflect the strengths of our proprietary GalXC technology platform."

"With a strong balance sheet and GalXC POC data in hand, we continue our progression into a rare disease company with the goal of developing a series of highly differentiated, subcutaneously delivered RNAi-based therapeutics to treat a range of rare diseases. To this end, we have made the strategic decision to develop our second, undisclosed rare disease candidate internally and will no longer seek a risk-sharing partner for this program prior to entry into the clinic. We intend to submit regulatory filings to initiate clinical trials for this undisclosed rare disease candidate in the first half of 2019."

Third Quarter 2018 and Recent Corporate Highlights

- Achieved clinical POC for DCR-PHXC in the ongoing PHYOX Phase 1 clinical trial in primary hyperoxaluria (PH). Initial findings demonstrate a clinically significant and meaningful reduction of mean maximal 24-hour urinary oxalate levels (defined as >30% reduction compared to baseline) in all patients following a single-dose administration in adults with PH type 1 (PH1) and PH type 2 (PH2).
 - Interim Phase 1 clinical data for DCR-PHXC in 25 normal healthy volunteers (NHVs) and 12 adult participants with PH, including both PH1 (n=11) and PH2 (n=1), were presented during the late-breaking clinical trials poster session at the American Society of Nephrology Kidney Week 2018 annual meeting.
 - The results (as of October 1, 2018) show that a single dose of 3.0 mg/kg 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 PH participants dosed at this level, including a mean maximal reduction in 24-hour urinary oxalate of 65% for the cohort. A single 1.5 mg/kg dose led to near-normalization (defined as 24-hour excretion < 0.6 and > 0.46 mmol) in three out of four PH1 participants dosed at this level, and led to a mean maximal reduction in urinary oxalate of 50% in the five PH participants dosed at that level, including one PH2 participant. Follow-up is ongoing.
 - Preliminary results also show that DCR-PHXC is safe and well-tolerated in NHVs and participants with PH. As of a data cut on October 1, 2018, 27 participants have been dosed with DCR-PHXC. No severe or serious adverse events have occurred in the PHYOX trial, and there have been no clinically significant changes in electrocardiography (ECG), vital signs, and laboratory or hematology values. Five (19%) participants experienced mild-to-moderate injection site reactions, all of which were transient and resolved without intervention within 24 to 72 hours.
 - Dicerna is on track to initiate a multi-dose, Phase 2/3 registration trial in the first quarter of 2019, pending regulatory feedback.
- Entered into 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 cardio-metabolic disease, neurodegeneration, and pain.
 - Dicerna and Lilly will seek to use Dicerna's GalXC RNAi technology platform to progress new drug targets toward clinical development and commercialization. In addition, the companies will collaborate to extend the GalXC RNAi platform technology to non-liver tissues, including neural tissues. The agreement contemplates collaboration on more than 10 targets in total.
 - Under the terms of the agreement, Dicerna will receive an upfront payment of \$100.0 million, with Lilly making a concurrent \$100.0 million equity investment at a premium in Dicerna. Dicerna is also eligible to potentially receive up to approximately \$350.0 million per target in development and commercialization milestones. The agreement also includes potential royalties on product sales.

- The agreement is subject to clearance under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary closing conditions.
- Entered into a collaborative research and license agreement with Alexion Pharmaceuticals (Alexion) for the joint discovery and development of RNAi therapies for complement-mediated diseases.
 - The agreement is for development and commercial rights for two of Dicerna's pre-clinical GalXC RNAi programs directed to complement pathway targets, with options for the discovery and development of candidates against two additional complement pathway targets.
 - Under the terms of the agreement, Dicerna received an upfront payment of \$22.0 million, with Alexion making a concurrent \$15.0 million equity investment at a premium in Dicerna. The collaboration agreement also provides for potential additional payments to Dicerna for option fees, development fees and sales milestones of up to \$600.0 million. The agreement also includes potential royalties on product sales.
- Strengthened balance sheet with an equity financing yielding gross proceeds of \$115.0 million.
- Filed Clinical Trial Application (CTA) for DCR-HBVS, in development for the treatment of chronic hepatitis B virus (HBV), with the New Zealand Medicines and Medical Devices Safety Authority (MedSafe) and the Health and Disability Ethics Committee (HDEC) to initiate a Phase 1 clinical trial in healthy volunteers and patients with chronic HBV.
 - Pending CTA approval, Dicerna intends to initiate the clinical study in early 2019.
 - The primary objective of the study is to evaluate the safety and tolerability of DCR-HBVS in healthy volunteers and patients with non-cirrhotic chronic HBV. Secondary objectives are to characterize the pharmacokinetic profile and to evaluate preliminary pharmacodynamics and antiviral efficacy on plasma levels of hepatitis B surface antigen (HBsAg) and HBV in blood.
- Continued development of Dicerna's undisclosed program for a rare disease involving the liver, currently in IND-enabling studies.
 - Given the expanded capital resources provided by the recent financing and the collaboration and licensing agreements with Lilly and Alexion, Dicerna intends to continue internal development of this program and is no longer seeking a risk-sharing collaborator prior to entry into the clinic.
 - Dicerna expects to submit regulatory filings to initiate clinical trials in the first half of 2019.
- Strengthened board of directors with the appointments of J. Kevin Buchi, former chief executive officer of Cephalon, Inc. and TetraLogic Pharmaceuticals Corp., and Cynthia Smith, former chief commercial officer of ZS Pharma, Inc.

Upcoming Regulatory and Clinical Milestones

- Initiate a multi-dose, Phase 2/3 registration trial of DCR-PHXC in the first quarter of 2019, pending regulatory feedback.
- Initiate Phase 1 clinical trial of DCR-HBVS in early 2019, pending regulatory approvals, and file additional regulatory clearances.
- Submit regulatory filings for undisclosed rare disease program to initiate clinical trials in the first half of 2019.

Financial Condition and Operating Results

- **Cash Position** – As of September 30, 2018, Dicerna had \$180.4 million in cash, cash equivalents and held-to-maturity investments, as compared to \$113.7 million in cash, cash equivalents and held-to-maturity investments as of December 31, 2017. Additionally, the Company had \$0.7 million of restricted cash equivalents as of September 30, 2018 and December 31, 2017, which reflects collateral securing the Company's operating lease obligation.
- **Revenue** – Dicerna recognized \$1.5 million and \$4.6 million of revenue associated with the Boehringer Ingelheim (BI) Agreement, during the three and nine months ended September 30, 2018, respectively. The revenue represents the periodic amortization of a non-refundable upfront payment of \$10.0 million for the first target and \$0.3 million in reimbursable third-party research expenses which are billable to BI. Dicerna expects to recognize the remainder of the initial transaction price on a straight-line basis through June 30, 2019. Dicerna does not expect to generate any product revenue for the foreseeable future.
- **Research and Development (R&D) Expenses** – R&D expenses were \$11.7 million and \$31.9 million for the three and nine months ended September 30, 2018, respectively, as compared to \$8.5 million and \$26.3 million for the same periods in 2017. The increase was primarily due to direct R&D expenses for both the three and nine months ended September 30, 2018 compared to the three and nine months ended September 30, 2017.

The increase in direct R&D expenses in the three months ended September 30, 2018 was primarily due to an increase in clinical study costs, reflecting increased activities associated with Dicerna's DCR-PHXC program, and an increase in consulting costs associated with various clinical development activities. The increase in R&D expenses was also impacted by higher employee-related expenses associated with increased headcount necessary to support the Company's growth, partially offset by lower platform-related expenses.

The increase in direct R&D expenses in the nine months ended September 30, 2018 was primarily due to higher clinical study costs due to increased activities associated with Dicerna's DCR-PHXC program and an increase in toxicology

studies for other product candidates. The increase in R&D expenses was also due to higher employee-related expenses associated with increased headcount necessary to support the Company's growth, partially offset by lower platform-related expenses.

Dicerna expects its overall R&D expense to continue to increase during the fourth quarter of 2018 and for the foreseeable future, primarily as the Company completes clinical manufacturing activities, advances pre-clinical 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 \$5.4 million and \$14.4 million for the three and nine months ended September 30, 2018, respectively, as compared to \$4.1 million and \$12.3 million for the same periods in 2017. The increase is predominantly related to increases in consulting costs, board of directors' compensation, and corporate legal expenses.
- **Litigation Expenses** – Litigation expenses for the nine months ended September 30, 2018, solely related to the litigation with Alnylam, increased predominantly due to \$3.7 million and \$24.7 million of settlement expenses recorded in the three and nine months ended September 30, 2018. The Company incurred \$3.7 million of litigation settlement expense in the three months ended September 30, 2018 in connection with the expected acceleration of the previously disclosed \$13.0 million payable to Alnylam resulting from the signing of the Lilly and Alexion collaboration agreements, and also incurred \$21.0 million of litigation settlement expense in the three months ended June 30, 2018 upon signing of the settlement agreement with Alnylam.
- **Net Loss Attributable to Common Stockholders** – Net loss attributable to common stockholders was \$19.0 million and \$70.2 million for the three and nine months ended September 30, 2018, respectively, as compared to \$19.1 million and \$57.3 million for the same periods in 2017. The decrease for the three months ended September 30, 2018 was driven by the \$4.1 million dividend on the redeemable convertible preferred stock for the three months ended September 30, 2017, offset by a \$3.2 million increase in research and development expenses and a \$1.2 million increase in general and administrative expenses. The increase in net loss attributable to common stockholders for the nine months ended September 30, 2018 was primarily due to a one-time increase of \$23.0 million in litigation expense, all of which related to the Alnylam litigation, partially offset by the \$12.9 million dividend on the redeemable convertible preferred stock recorded in the nine months ended September 30, 2017.

For more detailed information and analysis, see Dicerna's Quarterly Report on Form 10-Q for the quarter ended September 30, 2018, which was filed with the SEC on November 5, 2018.

Guidance

Dicerna had cash, cash equivalents, and held-to-maturity investments of approximately \$180.4 million as of September 30, 2018. The Company has received proceeds from the Alexion collaborative research and license agreement, and, combined with the expected proceeds from the upfront cash payment and equity investment from the Lilly collaborative research and license agreement (subject to the closing of the transactions contemplated by the Lilly agreement), expects to receive approximately \$224.0 million in net proceeds. Dicerna believes that its current cash, cash-equivalents and held-to-maturity investments, in addition to the upfront cash and equity investments from the Lilly and Alexion collaborative research and license agreements, will be sufficient to fund the execution of its current clinical and operating plan beyond 2020, which includes advancing DCR-PHXC through late-stage clinical development and regulatory filing, initiating proof-of-concept studies of DCR-HBVS in participants with HBV, and advancing the Company's undisclosed rare disease program into clinical development. This estimate assumes no new funding from additional collaboration agreements or from external financing events and no significant unanticipated changes in costs and expenses.

Conference Call

Management will host a conference call at 4:30 p.m. ET today to review Dicerna's third quarter 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 9588488 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 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 9588488. 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, viral infectious diseases, chronic liver diseases, and cardiovascular 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. The Company has strategic collaborations with Boehringer Ingelheim, Eli Lilly and Company, and Alexion Pharmaceuticals. 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, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular 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 pre-clinical data. The benefits seen in pre-clinical 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, including, for example, Dicerna's expected timeline and plans for development of DCR-PHXC, DCR-HBVS and other pipeline programs, expectations related to the collaborations with BI, Lilly and Alexion including the potential for additional payments, the closing of the Lilly transactions, expectations for discussions and possible opportunities with potential partners and collaborators, and guidance related to the anticipated duration and usage of current cash and cash equivalents. 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. The process by which an early stage platform such as GalXC could potentially lead to an approved product is long and subject to highly significant risks, particularly with respect to pre-clinical research collaborations. The Lilly transactions are subject to the satisfaction of closing conditions. Applicable risks and uncertainties include risks relating to Dicerna's clinical and pre-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 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)

	September 30, December 31,	
	2018	2017
Cash and cash equivalents	\$ 46,399	\$ 68,789
Held-to-maturity investments	\$ 133,980	\$ 44,889
Total assets	\$ 185,508	\$ 121,002
Total liabilities	\$ 29,128	\$ 19,916
Total stockholders' equity	\$ 156,380	\$ 101,086

Dicerna Pharmaceuticals, Inc.

Consolidated Statements of Operations

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue from collaborative arrangements	\$ 1,545	\$ —	\$ 4,635	\$ —
Operating expenses:				
Research and development	11,695	8,527	31,927	26,338
General and administrative	5,354	4,137	14,449	12,324
Litigation expense	3,694	2,548	29,122	6,157
Total operating expenses	20,743	15,212	75,498	44,819
Loss from operations	(19,198)	(15,212)	(70,863)	(44,819)
Other income (expense):				
Interest income	401	179	1,020	360
Interest expense	(223)	—	(399)	—
Total other income, net	178	179	621	360
Net loss	(19,020)	(15,033)	(70,242)	(44,459)
Dividends on redeemable convertible preferred stock	—	(4,111)	—	(6,733)
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	—	—	(6,144)
Net loss attributable to common stockholders	\$ (19,020)	\$ (19,144)	\$ (70,242)	\$ (57,336)

Net loss per share attributable to common stockholders — basic and diluted	\$ (0.35) \$ (0.92) \$ (1.32) \$ (2.76)
Weighted average common shares outstanding — basic and diluted	54,799,644	20,841,728	53,037,516	20,809,372	

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