

Reason for report:

FLASH NOTE

## DICERNA PHARMACEUTICALS, INC.

### DCR-PH1 Data Presented at Conference Confirm Preclinical Profile and Rationale

• **Bottom Line:** DRNA today announced the presentation of preclinical data on DCR-PH1, its RNAi based therapy for primary hyperoxaluria type 1 (PH1) at the International Primary Hyperoxaluria Workshop in Chicago. The data in our view confirmed the preclinical profile and scientific rationale for DCR-PH1. We continue to view DRNA as uniquely positioned in the therapeutic RNAi space, and we believe the company's pipeline strategy focused on rare orphan diseases and genetically defined cancers will pay off for investors longer term. Reiterate OP rating.

• **Minor updates presented at International PH Workshop confirm preclinical data and scientific rationale for DCR-PH1, in our view.** Mouse data presented by DRNA's collaborator Dr. Eduardo Salido now included data showing HAO1 knock-down data over 6 weeks following a single IV dose of DCR-PH1 vs. 4 weeks shown previously. Recall, the data showed sustained knock-down over a time period of at least 30 days (80% KD). Key derisking for DRNA's platform will be first-in-man data in 2H15, in our view.

• **Case study confirms DRNA's therapeutic strategy for PH1.** In addition, a case study of an asymptomatic child who has an AGT1 and a loss of function HAO1 mutation and increased urine glycolate excretion was accepted for publication in a scientific journal. We believe this case study confirms DRNA's therapeutic approach for PH1. Recall PH1 is caused by loss-of-function mutations in AGT1. DCR-PH1 is targeting knock-down of HAO1 (mimicking the genetic constellation of the child) which is an alternative metabolic enzyme of molecular pathway. HAO1 knock-down (or natural loss of function) results in urinary excretion of glycolic acid, a metabolic precursor of oxalate, thus avoiding its accumulation in kidneys.

• **Key derisking events expected in 2H15.** DRNA expects Phase I clinical data in 2H15 for its first two proprietary pipeline drugs, DCR-PH1 and DCR-MYC, which we believe could serve as proof-of-concept for these products and also derisk DRNA's platform, in particular with respect to successful human delivery and target knock-down efficiency. In 2015 DRNA also plans to expand its pipeline with one additional drug targeting a genetically defined disease involving the liver.

• **We continue to believe DRNA is uniquely positioned in the therapeutic RNAi space.** DRNA is one of only a few companies that have developed their own proprietary RNAi platform sidestepping key patents that represent high barriers to entry. We also believe DRNA's payloads are generally more potent than traditional siRNAs and allow conjugation of targeting agents for potential subcutaneous and tissue-targeted delivery. We thus believe DRNA is positioned well to develop a high-value proprietary pipeline and to close potential product development partnerships.

• **Next up: IND filing for DCR-PH1 expected in 1Q15.**

#### Key Stats:

(NASDAQ:DRNA)

|                                       |                 |
|---------------------------------------|-----------------|
| <b>S&amp;P 600 Health Care Index:</b> | <b>1,320.61</b> |
| <b>Price:</b>                         | <b>\$22.57</b>  |
| 52 Week High:                         | \$46.00         |
| 52 Week Low:                          | \$15.00         |
| Shares Outstanding (mil):             | 17.8            |
| Market Capitalization (mil):          | \$401.7         |

## Disclosures Appendix

### Analyst Certification

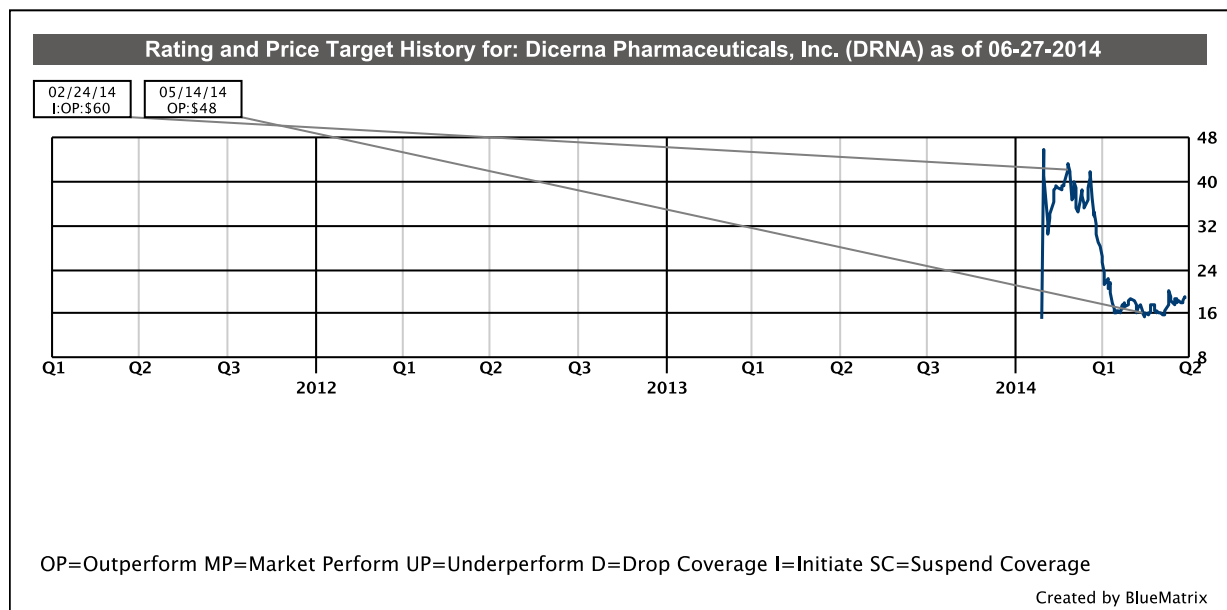
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

Our \$48 price target for DRNA shares in 12 months is based on a discounted cash flow (DCF) sum-of-parts analysis. Based on our DCF analysis, we attribute \$25/share to the pipeline, \$1/share to the KHK partnership, \$15/share to the platform, and the rest to net cash expected in one year. We use a 15% discount rate for probability of success-weighted pipeline products and a 15% discount rate for the DsiRNA technology platform. We probability-weight the MYC program at 20% and the PH1 program at 25% probability-of-success. We probability-weight the KRAS program at 15% POS and the second undisclosed candidate at 10% POS. We assume DRNA to receive a 9% royalty on worldwide sales for products under the KHK partnership. We value DRNA's technology platform using the assumption it will generate one additional new DsiRNA product candidate per year starting in 2015E. We discount assumed future profits and losses back using a 15% discount rate.

### Risks to Valuation

DRNA faces significant clinical and regulatory risks since all of its product candidates are currently in development. DRNA specifically also faces clinical development risk since none of its products have been tested in humans, and the company is developing first-in-class RNAi-based drugs with a novel proprietary delivery mechanism. In addition to that, DRNA's product candidates address new, clinically invalidated targets. Similar to many other developmental stage Biopharma companies, DRNA also faces manufacturing, competitive, commercial, regulatory, and safety risks, as well as risks to its intellectual property. In addition, DRNA faces financing risk dilutive to shareholders since we don't believe the company will be profitable for the foreseeable future. We see additional risks for investors since the company is closely held and substantially all of DRNA's outstanding shares are not subject to lock-up agreements in connection with its IPO.



| Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14 |       |         |                       |         |
|---|-------|---------|-----------------------|---------|
| Rating  | Count | Percent | IB Serv./Past 12 Mos. |         |
|   |       |         | Count                 | Percent |
| BUY [OP]  | 131   | 68.23   | 46                    | 35.11   |
| HOLD [MP]   | 61    | 31.77   | 3                     | 4.92    |
| SELL [UP]   | 0     | 0.00    | 0                     | 0.00    |

## Explanation of Ratings

**Outperform (Buy):** We expect this stock to outperform its benchmark over the next 12 months.

**Market Perform (Hold/Neutral):** We expect this stock to perform in line with its benchmark over the next 12 months.

**Underperform (Sell):** We expect this stock to underperform its benchmark over the next 12 months. The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

## Important Disclosures

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

**In the past 12 months, the Firm has received compensation for providing investment banking services to Dicerna Pharmaceuticals, Inc. .**

**Leerink Partners LLC makes a market in Dicerna Pharmaceuticals, Inc.**

**Leerink Partners LLC has acted as the manager for a public offering of Dicerna Pharmaceuticals, Inc. in the past 12 months.**

**©2014 Leerink Partners LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.**

## Leerink Partners LLC Equity Research

|  |                               |                |                                   |
|--|-------------------------------|----------------|-----------------------------------|
| <b>Director of Equity Research</b>                   | <b>John L. Sullivan, CFA</b>  | (617) 918-4875 | john.sullivan@leerink.com         |
| <b>Associate Director of Research</b>                | <b>Alice C. Avanian, CFA</b>  | (617) 918-4544 | alice.avanian@leerink.com         |
| <b>Healthcare Strategy</b>                           | <b>John L. Sullivan, CFA</b>  | (617) 918-4875 | john.sullivan@leerink.com         |
|  | <b>Alice C. Avanian, CFA</b>  | (617) 918-4544 | alice.avanian@leerink.com         |
| <b>Biotechnology</b>                                 | <b>Howard Liang, Ph.D.</b>    | (617) 918-4857 | howard.liang@leerink.com          |
|  | <b>Joseph P. Schwartz</b>     | (617) 918-4575 | joseph.schwartz@leerink.com       |
|  | <b>Marko Kozul, M.D.</b>      | (415) 905-7221 | marko.kozul@leerink.com           |
|  | <b>Michael Schmidt, Ph.D.</b> | (617) 918-4588 | michael.schmidt@leerink.com       |
|  | <b>Gena Wang, Ph.D., CFA</b>  | (212) 277-6073 | gena.wang@leerink.com             |
|  | Jonathan Chang, Ph.D.         | (617) 918-4015 | jonathan.chang@leerink.com        |
|  | Paul Matteis                  | (617) 918-4585 | paul.matteis@leerink.com          |
|  | Richard Goss                  | (617) 918-4059 | richard.goss@leerink.com          |
| <b>Life Science Tools and Diagnostics</b>            | <b>Dan Leonard</b>            | (212) 277-6116 | dan.leonard@leerink.com           |
|  | Justin Bowers, CFA            | (212) 277-6066 | justin.bowers@leerink.com         |
| <b>Pharmaceuticals/Major</b>                         | <b>Seamus Fernandez</b>       | (617) 918-4011 | seamus.fernandez@leerink.com      |
|  | Ario Arabi                    | (617) 918-4568 | ario.arabi@leerink.com            |
|  | Aneesh Kapur                  | (617) 918-4576 | aneesh.kapur@leerink.com          |
| <b>Specialty Pharmaceuticals</b>                     | <b>Jason M. Gerberry, JD</b>  | (617) 918-4549 | jason.gerberry@leerink.com        |
| <b>Medical Devices, Cardiology &amp; Orthopedics</b> | <b>Danielle Antalffy</b>      | (212) 277-6044 | danielle.antalffy@leerink.com     |
|  | <b>Richard Newitter</b>       | (212) 277-6088 | richard.newitter@leerink.com      |
|  | Ravi Misra                    | (212) 277-6049 | ravi.misra@leerink.com            |
| <b>Healthcare Services</b>                           | <b>Ana Gupte, Ph.D.</b>       | (212) 277-6040 | ana.gupte@leerink.com             |
| <b>Healthcare Technology &amp; Distribution</b>      | <b>David Larsen, CFA</b>      | (617) 918-4502 | david.larsen@leerink.com          |
|  | Christopher Abbott            | (617) 918-4010 | chris.abbott@leerink.com          |
| <b>Sr. Editor/Supervisory Analyst</b>                | <b>Mary Ellen Eagan, CFA</b>  | (617) 918-4837 | maryellen.eagan@leerink.com       |
| <b>Supervisory Analysts</b>                          | Robert Egan                   |                | bob.egan@leerink.com              |
|  | Amy N. Sonne                  |                | amy.sonne@leerink.com             |
| <b>Editorial</b>                                     | Cristina Diaz-Dickson         | (617) 918-4548 | cristina.diaz-dickson@leerink.com |
| <b>Research Assistant</b>                            | Carmen Augustine              | (212) 277-6012 | carmen.augustine@leerink.com      |

**New York**  
299 Park Avenue, 21<sup>st</sup> floor  
New York, NY 10171  
(888) 778-1653

**Boston**  
**One Federal Street, 37<sup>th</sup> Floor**  
**Boston, MA 02110**  
**(800) 808-7525**

**San Francisco**  
201 Spear Street, 16<sup>th</sup> Floor  
San Francisco, CA 94105  
(800) 778-1164