

Reason for report:

**EARNINGS**

## DICERNA PHARMACEUTICALS, INC.

### First Clinical Study Set to Start on Schedule in 2Q; Updating Our Model

• **Bottom Line:** DRNA reported 2013 financial results today after market close. The company ended the year with \$47M in cash not including IPO proceeds of \$93M in 1Q14. Management expects to have sufficient cash to fund the company through 2016. The pipeline remains on track with initiation of Phase I for DCR-M1711 planned in 2Q14. We are updating our model to reflect 2013 results. **Reiterate Outperform rating and \$60 price target.**

• **DRNA continues to plan to initiate a Phase I of DCR-M1711** in non-hepatocellular carcinoma (non-HCC) tumors in the first half of 2014. The Phase I study will be a standard dose escalation trial to determine the maximum tolerated dose in non-HCC patients followed by an expansion cohort at the maximum tolerated dose. First in man data expected to be available in 2015, according to management. Recall, DCR-M1711 targets the MYC oncogene, which is up-regulated in a wide variety of tumor types.

• **DRNA continues to expect completion of optimization of DCR-PH1** and declare a clinical candidate for this program in 1H14, followed by manufacturing scale-up and good laboratory practice (GLP) toxicity studies before initiating clinical trials in 2015. Recall, DCF-PH1 targets glycolate oxidase in the liver. Glycolate oxidase knock-down reduces the production of oxalate which accumulates in kidney tissues of patients with Primary Hyperoxaluria (PH1), an ultra-orphan disease.

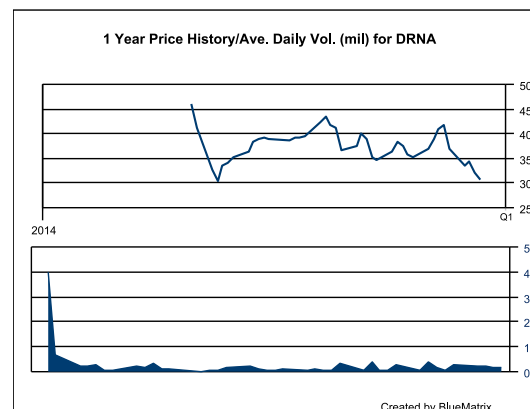
• **Management expects to disclose a third proprietary product candidate this year.** This Dicer substrate will also enter the clinic in 2015, according to management, and will either address a rare liver disease or a genetically defined cancer.

• **We continue to believe DRNA is uniquely positioned within the therapeutic RNAi landscape** in that it has developed its own proprietary RNAi platform based on Dicer substrates, which we believe are differentiated from other technologies. We believe two key value drivers for DRNA are first-in-man Phase I data expected in late 2015 from its two lead proprietary pipeline products. We believe that data would not only provide early biomarker proof-of-concept for DRNA's first two products but also de-risk DRNA's platform, which should be attractive for potential future partners.

#### Key Stats:

(NASDAQ:DRNA)

<b>S&amp;P 600 Health Care Index:</b>	<b>1,249.14</b>
<b>Price:</b>	<b>\$30.61</b>
Price Target:	\$60.00
Methodology:	Sum-of-the parts DCF analysis
52 Week High:	\$46.00
52 Week Low:	\$15.00
Shares Outstanding (mil):	17.8
Market Capitalization (mil):	\$544.9
Book Value/Share:	\$7.93
Cash Per Share:	\$7.90
Dividend (ann):	\$0.00
Dividend Yield:	0.0%

*Book Value: pro forma**Cash Per Share: includes proceeds from IPO*

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	--	--	0.0	0.0	0.0	--	--	(\$505.45)	(\$200.00)	(\$710.00)	NM
2014E - New	0.0	0.0	0.0	\$3.3	\$3.3	(\$0.51)	(\$0.51)	(\$0.51)	(\$0.32)	(\$1.84)	NM
2014E - Old	0.0	0.0	0.0	\$3.3	\$3.3	(\$0.51)	(\$0.51)	(\$0.51)	(\$0.33)	(\$1.87)	NM
2015E - New	--	--	--	--	\$1.1	--	--	--	--	(\$2.36)	NM
2015E - Old	--	--	--	--	\$1.1	--	--	--	--	(\$2.39)	NM

Source: Company Information and Leerink Partners LLC Research  
Revenues in millions.

GAAP EPS: Q1-Q3:13, 2013, pre-IPO.

## INVESTMENT THESIS

### **We rate Dicerna Pharmaceuticals (DRNA) Outperform with a \$60/share price target (PT).**

We believe DRNA is uniquely positioned within the therapeutic RNAi landscape in that it has developed its own proprietary RNAi platform based on Dicer substrates, which we believe are differentiated from other technologies. We believe two key value drivers for DRNA are first-in-man Phase I data expected in late 2015 from its two lead proprietary pipeline products. We believe that data would not only provide early biomarker proof-of-concept for DRNA's first two products but also de-risk DRNA's platform, which should be attractive for potential future partners.

**DRNA is uniquely positioned in the RNAi space.** DRNA is one of only a few companies that have been able to develop their own proprietary RNAi platform consisting of DsiRNA payloads and a proprietary delivery technology. DRNA's main differentiation from other RNAi technologies is that it is using Dicer substrates as therapeutic siRNA molecules, which allows DRNA to sidestep key patents that represent high barriers to entry in the RNAi space. In addition, we believe DRNA's payloads are generally more potent than traditional siRNAs and allow conjugation of targeting agents potentially allowing subcutaneous and tissue-targeted delivery in the near future. We thus believe DRNA is positioned well to close additional product development partnerships in the near future, which could serve as further validation of its platform.

**Proprietary pipeline focused on orphan diseases and oncology with major de-risking events in late 2015.** DRNA expects Phase I clinical data in 2H15 for its first two proprietary pipeline drugs, DCR-PH1 and DCR-M1711, which we believe could serve as early proof-of-concept for these products and also de-risk 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. DCR-PH1 is being developed to treat Primary Hyperoxaluria Type 1 (PH1), a rare, genetic disease of the liver with a strong scientific rationale, in our view. DCR-M1711 targets Myc that is being developed initially in liver cancer (HCC) but could potentially have activity across a large number of solid tumor indications. The potential of Myc as a therapeutic target has been recognized for decades, but the undruggable nature of Myc has been a significant barrier to traditional small molecule and antibody-based approaches.

## VALUATION

Our \$60 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 \$36/share to the pipeline, \$2/share to the KHK partnership, \$15/share to the platform, and the rest to net cash expected in one year. We use a 12% 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.

<b>DRNA Income Statement</b>	<b>2011</b>	<b>2012</b>	<b>1-3Q13</b>	<b>4Q13</b>	<b>2013</b>	<b>1Q14E</b>	<b>2Q14E</b>	<b>3Q14E</b>	<b>4Q14E</b>	<b>2014E</b>	<b>2015E</b>
Product revenue	-	-	-	-	-	-	-	-	-	-	-
License fee and research funding	2.5	-	-	-	-	-	-	-	-	-	-
Fees, milestones (p/w)	5.0	6.5	-	-	-	-	-	-	3.3	3.3	1.1
<b>Total Revenues</b>	<b>7.9</b>	<b>7.0</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>3.3</b>	<b>3.3</b>	<b>1.1</b>
COGS	-	-	-	-	-	-	-	-	-	-	-
R&D	10.7	11.6	7.4	4.2	11.6	7.0	7.0	7.0	7.0	28.0	33.0
SG&A	4.8	4.7	3.6	2.2	5.8	2.0	2.0	2.0	2.0	8.0	10.0
Total costs and expenses	15.5	16.3	10.9	6.4	17.4	9.0	9.0	9.0	9.0	36.0	43.0
<b>Operating Income (Loss)</b>	<b>(7.6)</b>	<b>(9.3)</b>	<b>(10.9)</b>	<b>(6.4)</b>	<b>(17.4)</b>	<b>(9.0)</b>	<b>(9.0)</b>	<b>(9.0)</b>	<b>(5.8)</b>	<b>(32.8)</b>	<b>(42.0)</b>
Income before taxes	(8.6)	(10.1)	(11.8)	(6.7)	(18.5)	(9.0)	(9.0)	(9.0)	(5.8)	(32.8)	(42.0)
Income tax expense	-	-	-	-	-	-	-	-	-	-	-
<b>Net income (Loss)</b>	<b>(8.6)</b>	<b>(10.1)</b>	<b>(11.8)</b>	<b>(6.7)</b>	<b>(18.5)</b>	<b>(9.0)</b>	<b>(9.0)</b>	<b>(9.0)</b>	<b>(5.8)</b>	<b>(32.8)</b>	<b>(42.0)</b>
Loss: accretion and dividends on rede	(4.1)	(4.1)	(2.4)	0.0	(2.4)	-	-	-	-	-	-
Net loss attributable to common stock	(12.7)	(14.2)	(14.2)	(6.7)	(20.9)	(9.0)	(9.0)	(9.0)	(5.8)	(32.8)	(42.0)
<b>EPS</b>	<b>(493)</b>	<b>(516)</b>	<b>(505)</b>	<b>(200)</b>	<b>(710)</b>	<b>(0.51)</b>	<b>(0.51)</b>	<b>(0.51)</b>	<b>(0.32)</b>	<b>(1.84)</b>	<b>(2.36)</b>
Shares outstanding	0.0	0.0	0.0	0.0	0.0	17.8	17.8	17.8	17.8	17.8	17.8

<b>BS &amp; CFS</b>	<b>2011</b>	<b>2012</b>	<b>1-3Q13</b>	<b>4Q13</b>	<b>2013</b>	<b>1Q14E</b>	<b>2Q14E</b>	<b>3Q14E</b>	<b>4Q14E</b>	<b>2014E</b>	<b>2015E</b>
Cash & investments	22.5	3.7	54.7	46.6	46.6	134.8	130.1	125.4	124.0	124.0	99.8
Debt	11.6	8.8	5.9	4.8	4.8	0.0	0.0	0.0	0.0	0.0	0.0

Change in Cash	(2.1)	(18.8)	51.0	(8.1)	42.9	88.2	(4.7)	(4.7)	(1.4)	77.4	(24.1)
<b>Cash from operations</b>	<b>(8.9)</b>	<b>(15.7)</b>	<b>(5.3)</b>	<b>(5.6)</b>	<b>(10.9)</b>	<b>(7.5)</b>	<b>(7.5)</b>	<b>(7.5)</b>	<b>(4.3)</b>	<b>(26.9)</b>	<b>(35.5)</b>
Net income (loss)	(8.6)	(10.1)	(11.8)	(6.7)	(18.5)	(9.0)	(9.0)	(9.0)	(5.8)	(32.8)	(42.0)
Share based comp	0.2	0.1	0.2	0.3	0.5	0.7	0.7	0.7	0.7	2.9	3.4
D&A	0.8	0.8	0.6	(0.0)	0.5	(0.0)	(0.0)	(0.0)	(0.0)	(0.1)	(0.1)
Other (Change in WC)	(1.3)	(6.5)	5.8	0.8	6.6	0.8	0.8	0.8	0.8	3.1	3.1
<b>Cash from investing</b>	<b>(0.5)</b>	<b>(0.1)</b>	<b>(0.2)</b>	<b>(0.2)</b>	<b>(0.4)</b>	<b>(0.2)</b>	<b>(0.2)</b>	<b>(0.2)</b>	<b>(0.2)</b>	<b>(0.9)</b>	<b>(0.9)</b>
CapEx	(0.5)	(0.1)	(0.2)	(0.2)	(0.4)	(0.2)	(0.2)	(0.2)	(0.2)	(0.9)	(0.9)
Acquisitions	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-
<b>Cash from financing</b>	<b>7.3</b>	<b>(3.0)</b>	<b>56.5</b>	<b>(2.3)</b>	<b>54.3</b>	<b>96.0</b>	<b>3.1</b>	<b>3.1</b>	<b>3.1</b>	<b>105.2</b>	<b>12.3</b>
Equity issue (buyback)	0.1	0.0	57.0	-	57.0	92.9	-	-	-	92.9	-
Debt issue (principal payment)	8.6	(3.0)	(0.1)	3.1	3.0	3.1	3.1	3.1	3.1	12.3	12.3
Other	(1.4)	-	(0.4)	(5.3)	(5.7)	-	-	-	-	-	-

Source: Company filings and Leerink Partners Estimates

DRNA Valuation (p/w)	POS	P/W NPV	Per Share
<b>Total</b>		<b>1,060</b>	<b>60</b>
<b>Pipeline</b>		<b>641</b>	<b>36</b>
MYC	20%	343	19
PH1	25%	298	17
<b>KHK Partnership</b>		<b>33</b>	<b>2</b>
KHK KRAS	15%	22	1
KHK Oncology 2	10%	11	1
<b>Platform</b>	10%	<b>263</b>	<b>15</b>
Cash in 1 year	100%	124	7

Source: Leerink Partners Estimates

<b>Product Candidate (proprietary)</b>	<b>Target</b>	<b>Event</b>	<b>Expected Timing</b>
DCR-PH1	HAO1	Initiate Phase I	1Q15
		Phase I data	4Q15
DCR-M1711	MYC	Initiate Phase I non-HCC tumors	2Q14
		Phase I data non-HCC	2015
		Initiate Phase I HCC (Asia)	2H14
		Phase I HCC data	2015
Undisclosed	Liver/orphan	Initiate Phase I	2015
<b>Product Candidate (KHK partnership)</b>	<b>Target</b>	<b>Event</b>	<b>Expected Timing</b>
Undisclosed	KRAS	Initiate Phase I	2015
		Phase I data	2016
Undisclosed	Cancer	Initiate Phase I	2016
		Phase I data	2017
<b>Partnerships</b>			<b>Expected Timing</b>
DCR-1711 ROW partnership			2014/2015
Addl development or platform deals			2014/2015

Source: SEC filings and Leerink Partners estimates

## Disclosures Appendix

### Analyst Certification

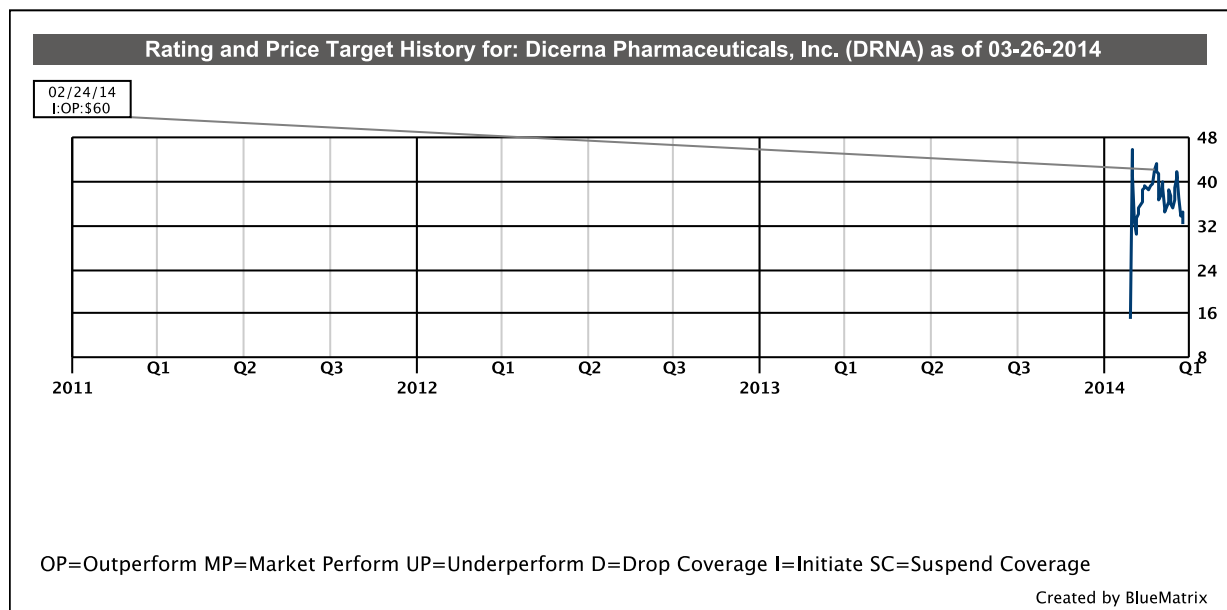
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

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Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	118	64.50	30	25.00
HOLD [MP]	65	35.50	2	3.00
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

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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.

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