December 16, 2014

OUTPERFORM

Reason for report:

FLASH NOTE

Michael Schmidt, Ph.D.
(617) 918-4588
Michael.Schmidt@Leerink.com

Jonathan Chang, Ph.D.
(617) 918-4015
Jonathan.Chang@Leerink.com

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DICERNA PHARMACEUTICALS, INC.

Positive Pipeline Updates at R&D Update Call

Key Stats:	(NASDAQ:DRNA)
S&P 600 Health Care Index:	1,372.50
Price:	$13.08
52 Week High:	$46.00
52 Week Low:	$8.00
Shares Outstanding (mil):	17.7
Market Capitalization (mil):	$231.5

• **Bottom Line:** DRNA hosted an R&D Update call today and provided several positive updates on its DsiRNA platform: (1) DRNA introduced new proprietary second generation "DsiRNA-EX" payloads with added functionality; (2) DRNA is currently optimizing four liver-targeted subcutaneously administered DsiRNA-EX-conjugates, the first of which will enter the clinic in 2016; (3) DRNA presented impressive first DCR-PH1 non-human primate (NHP) data supportive of once-monthly or less frequent dosing; (4) both clinical programs DCR-PH1 and DCR-MYC remain on track for first data readouts in 4Q15. We continue to believe DRNA has an attractive platform and strategy, proven management team, and a strong cash position, which we believe should suffice to advance the pipeline through several key value inflection points. With an EV of only $116M, we believe DRNA shares are significantly undervalued. **Reiterate Outperform rating.**

• **DRNA's DsiRNA platform evolves with important advances adding functionality and subcutaneous dosing capability to its RNAi payloads.** DRNA introduced its new proprietary "DsiRNA-EX" payload design and DsiRNA-EX-conjugate payloads enabling subcutaneous delivery. DCR-PH1 is the first pipeline candidate which incorporates the proprietary DsiRNA-EX payload in which one of the two RNA strands (guide strand or passenger strand) carries an extension. DRNA presented data in peripheral blood mononuclear cells suggesting that DsiRNA-EX payloads have the potential for decreased immunotoxicity while maintaining equal potency compared to DRNA's 1st generation DsiRNA RNAi triggers. Importantly, the DsiRNA-EX platform can also be configured for subcutaneous conjugate-mediated liver-targeted delivery.

• **DRNA is currently optimizing DsiRNA-EX-conjugates for four therapeutic liver targets, the first of which will enter the clinic in 2016.** Mouse data presented suggested 50% knock-down rates after a single 2mg/kg subcutaneous injection indicating DRNA may have already achieved a viable therapeutic range. The four new targets as well as the type of conjugation and stabilization chemistry used were not disclosed yet for competitive reasons and given that patent prosecution is still in early stages. We view DRNA's successful efforts to evolve its platform positively, which we think is necessary to remain competitive in the RNAi therapeutics landscape. Given DRNA's secrecy around its next product candidates, we speculate that they could possibly represent new first-in class targets not yet pursued by others.

• **Impressive first DCR-PH1 non-human primate (NHP) data supportive of once-monthly or less frequent dosing.** DRNA showed that a single dose of 0.3mg/kg DCR-PH1 achieved up to 93% knockdown of HAO1 (84% mean knockdown) at day 4 and up to 86% knockdown (68% mean knockdown) at day 29. This was achieved using a proprietary DsiRNA-EX payload formulated with TKMR's (MP) lipid nanoparticle delivery system. Recall DCR-PH1 targets HAO1, a metabolic enzyme expressed in the liver as potential disease-modifying therapy for primary hyperoxaluria Type I (PH1). We believe the data are supportive of an

infrequent dosing regimen, potentially with a dosing interval of one month or longer.

• **DRNA is planning to initiate a natural history study of PH1 in 1Q15.** This study will enroll 50-75 genetically confirmed PH1 patients in the US and EU (excluding patients on dialysis) and will aim to characterize the natural history of PH1, changes in oxalate and glycolate levels and renal function over time, and event rates associated with PH1. This study will provide a historical group for comparison to data from clinical trials. We view the planned natural history study positively as it would provide guidance on the eventual pivotal trial study design and facilitate patient enrollment into future DCR-PH1 trials.

• **DCR-PH1 Phase I study is expected to initiate in 2H15.** The trial will include both a single ascending dose (SAD) portion and a multiple ascending dose (MAD) portion, both in PH1 patients. The dosing regimen will be IV infusion over 30-60 minutes. Data from the SAD portion of the study are expected by YE15, and data from both the SAD and MAD portions of the study should be available in 2016.

• **DCR-MYC development is on track with initial clinical data from the first trial expected by YE15.** DCR-MYC-101 is a Phase I study in advanced solid tumors, myeloma, or lymphoma that initiated in 2Q14. Enrollment is ongoing at two US sites. DCR-MYC-102 is a Phase Ib/II study in hepatocellular carcinoma (HCC) patients and is expected to initiate in Dec 2014 in the US and Asia. The dosing regimen for both trials will be intravenous infusion over 2 hours weekly for two weeks, followed by a 1 week break. FDG-PET scans will be utilized for early detection of response.

Disclosures Appendix

Analyst Certification

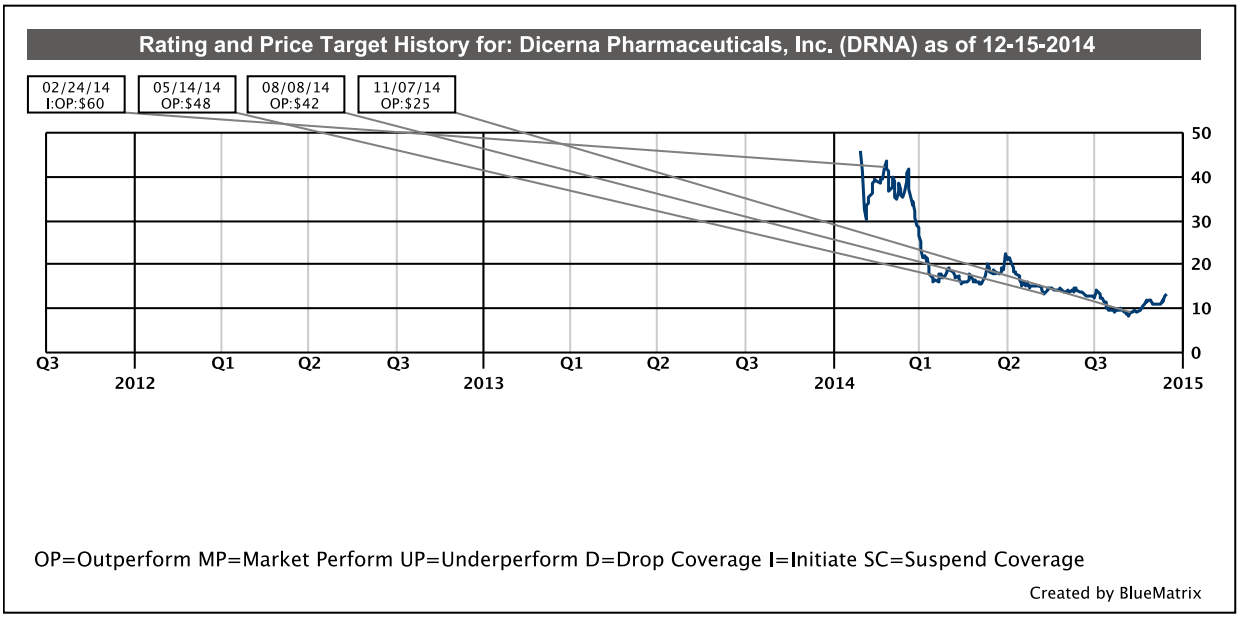
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

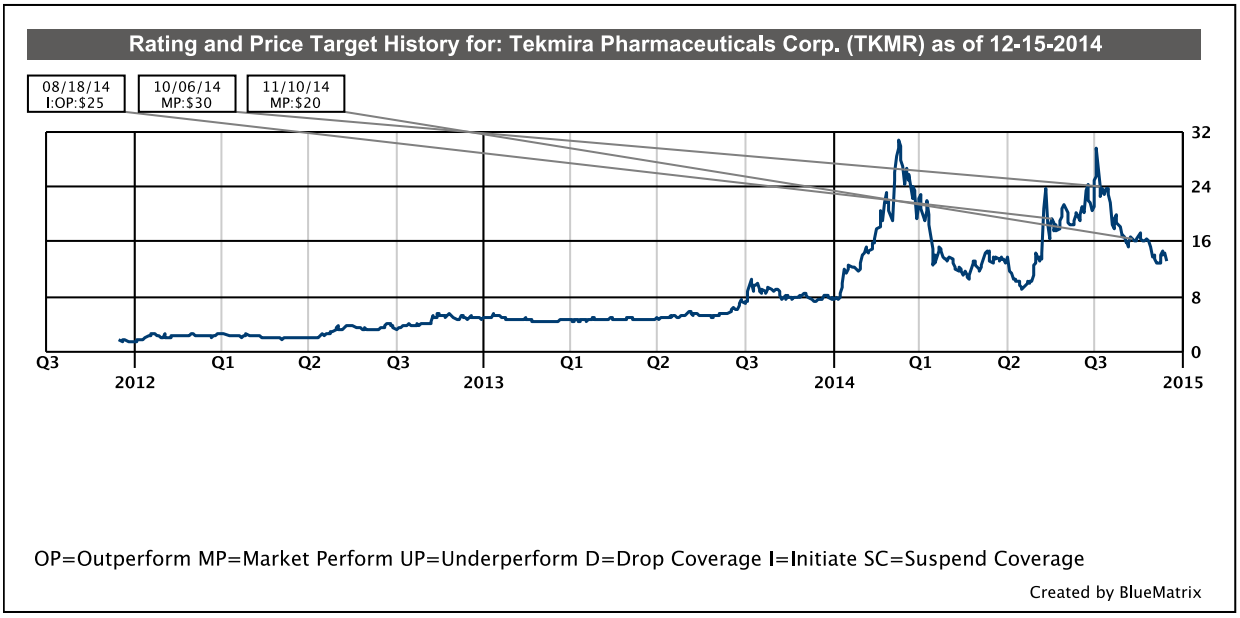
Valuation

Our $25 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 $18/share to the pipeline and the rest to net cash. We use a 15% discount rate for probability of success-weighted pipeline products. We probability-weight the MYC program at 20% and the PH1 program at 25% probability-of-success. The KRAS program partnered with KHK and additional product candidates generated by DRNA's platform are sources of upside to our valuation.

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.




Rating and Price Target History for: Tekmira Pharmaceuticals Corp. (TKMR) as of 12-15-2014
08/18/14
I:OP:$25
10/06/14
MP:$30
11/10/14
MP:$20
32
24
16
8
0
Q3
2012
Q1
Q2
Q3
2013
Q1
Q2
Q3
2014
Q1
Q2
Q3
2015
OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage
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Distribution of Ratings/Investment Banking Services (IB) as of 09/30/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	138	69.30	51	37.00
HOLD [MP]	61	30.70	2	3.30
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. and Tekmira Pharmaceuticals Corp. .

Leerink Partners LLC makes a market in Dicerna Pharmaceuticals, Inc. and Tekmira Pharmaceuticals Corp.

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

Leerink Partners LLC has acted as the manager for a public offering of Tekmira Pharmaceuticals Corp. in the past 12 months.

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Leerink Partners LLC Equity Research

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	**Alice C. Avanian, CFA**	(617) 918-4544	alice.avanian@leerink.com
Healthcare Strategy	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Biotechnology	**Howard Liang, Ph.D.**	(617) 918-4857	howard.liang@leerink.com
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
Life Science Tools and Diagnostics	**Dan Leonard**	(212) 277-6116	dan.leonard@leerink.com
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink.com
Pharmaceuticals/Major	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink.com
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink.com
Specialty Pharmaceuticals	**Jason M. Gerberry, JD**	(617) 918-4549	jason.gerberry@leerink.com
	Derek C. Archila	(617) 918-4851	derek.archila@leerink.com
Medical Devices, Cardiology & Orthopedics	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink.com
	Puneet Souda	(212) 277-6091	puneet.souda@leerink.com
	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Ravi Misra	(212) 277-6049	ravi.misra@leerink.com
Healthcare Services	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink.com
	Matthew Milask	(212) 277-6084	matthew.milask@leerink.com
Healthcare Technology & Distribution	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink.com
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Digital Health	**Steven Wardell**	(617) 918-4097	steven.wardell@leerink.com
Sr. Editor/Supervisory Analyst	**Mary Ellen Eagan, CFA**	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink.com
Research Associate	Carmen Augustine	(212) 277-6012	carmen.augustine@leerink.com

New York
299 Park Avenue, 21st floor
New York, NY 10171
(888) 778-1653

Boston
One Federal Street, 37th Floor
Boston, MA 02110
(800) 808-7525

San Francisco
201 Spear Street, 16th Floor
San Francisco, CA 94105
(800) 778-1164