

COMPANY NOTE

Estimate Change

USA | Healthcare | Biotechnology

November 7, 2014

Jefferies

Dicerna Pharmaceuticals (DRNA) 3Q14 Update – Critical Clinical Pipeline Data On Track For 2015

Key Takeaway

During its 3Q call, DRNA reiterated its focus on the 2015 initiation of Phase 1 clinical trials for both DCR-PH1 and DCR-MYC with topline data for both expected by year end 2015. We believe DRNA has adequate cash to fund operations through these key data readouts and maintain our \$48 price target.

We Continue To Expect DCR-PH1 Data In 2015. With the Phase 1 trial expected to begin in 2015, we continue to expect a 50% reduction in urinary oxalate to be an acceptable endpoint for approval, based on expert commentary at the International Primary Hyperoxaluria Workshop in June. We expect proof-of-concept results by 2H15. DRNA held an advisory board meeting with thought leaders who made it clear that reduction in oxalate levels would be beneficial for all genotypes.

DRNA Addresses ALNY Competition. On its call, DRNA addressed Alnylam's (ALNY, NC) recently presented RNAi-based therapeutic program for PH1. As a reminder, in addition the DCR-PH1 presentation at the Olfonucleotide Therapeutics Society meeting in October, ALNY presented rodent data for its ALN-GO1, an ESC-GalNAc conjugate siRNA that targets glycolate oxidase (GO) that was shown to decrease urinary oxalate >70%. ALNY will identify an ALN-GO1 candidate in mid-2015 and file with the FDA to begin trials in 2016, giving DRNA roughly a year lead. Nevertheless, we see this fast-follower as a potentially legitimate threat, given ALNY's track record and subcutaneous administration. Separately on the competitive front, we are looking for Oxthera's Oxabact to have data in December, but expect DCR-PH1 to show better efficacy.

DCR-MYC Program On Track. The first dose-finding Phase 1 trial in solid tumors, multiple myeloma, and lymphomas began in April, and DRNA noted that they were pleased with the enrollment. Having escalated through many doses, the company notes it has yet to hit a maximum tolerated dose (MTD), which along with determining pharmacokinetics and anti-tumor activity of DCR-MYC, is the goal of the trial. DRNA still expects topline data by 2H15. A Phase 1 trial in advanced hepatocellular carcinoma (HCC) is still expected to begin in Asia in 2H14. Given its stage of development, we view this program as high risk.

Financials. DRNA reiterated its expectation that to have sufficient cash to fund operations through 2016. As of September, DRNA had a cash position of \$112m.

Valuation/Risks

We derive our \$48 price target on a DCF based sum-of-parts valuation. Risks include: clinical, regulatory, and commercial.

USD	Prev.	2014E	Prev.	2015E	Prev.	2016E	Prev.	2017E
Rev. (MM)	--	0.0	--	0.0	--	--	--	--
EPS								
Mar	--	(0.56)A	--	--	--	--	--	--
Jun	--	(0.51)A	--	--	--	--	--	--
Sep	(0.53)	(0.54)A	--	--	--	--	--	--
Dec	--	(0.56)	--	--	--	--	--	--
FY Dec	(2.15)	(2.16)	--	(2.48)	--	--	--	--
FY P/E		NM		NM				

Limited historical data due to recent company IPO

BUY

Price target \$48.00

Price \$8.14

Financial Summary

Net Debt (MM):	(\$111.9)
Cash (MM):	\$111.9

Market Data

52 Week Range:	\$46.00 - \$8.00
Total Entprs. Value (MM):	\$30.6
Market Cap. (MM):	\$142.5
Insider Ownership:	43.3%
Institutional Ownership:	38.3%
Shares Out. (MM):	17.5
Float (MM):	8.8
Avg. Daily Vol.:	171,411

Thomas Wei *

Equity Analyst

(212) 284-2326 twei@jefferies.com

Timothy Chou *

Equity Associate

(212) 284-2571 tchou@jefferies.com

Shaunak Deepak *

Equity Analyst

(212) 284-2020 sdeepak@jefferies.com

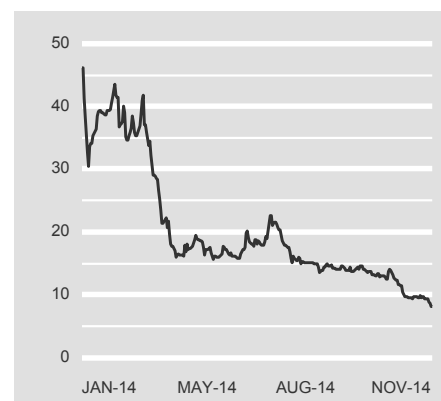
Rebecca Forest *

Equity Associate

(212) 284-2170 rforest@jefferies.com

* Jefferies LLC

Price Performance



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DRNA: Historical and Projected Revenue and Earnings

December 31 Fiscal Year (\$000s, except per share)	2012	2013	1Q14A	2Q14A	3Q14A	4Q14E	2014	2015	2016	2017	2018	2019	2020	2021	2022
End User Sales															
U.S. PHL Sales		0	0	0	0	0	0	0	0	15,581	63,225	106,783	154,514	201,448	245,079
Ex-U.S. PHL Sales		0	0	0	0	0	0	0	0	22,073	88,885	155,040	224,326	293,117	355,972
VWVPHL Sales		0	0	0	0	0	0	0	0	37,654	153,120	261,823	378,890	494,565	601,051
VWVDCR-MIL711 Sales		0	0	0	0	0	0	0	0	0	0	0	66,150	667,229	1,829,961
Dicerna Reported Revenue															
U.S. PHL Sales		0	0	0	0	0	0	0	0	15,581	63,225	106,783	154,514	201,448	245,079
Ex-U.S. PHL Royalties		0	0	0	0	0	0	0	0	4,415	19,974	36,512	57,298	77,935	96,792
DCR-MIL711 Royalties		0	0	0	0	0	0	0	0	0	0	9,922	100,084	274,494	451,188
Milestones		0	0	0	0	0	0	0	50,000	125,000	25,000	25,000	125,000	75,000	75,000
Other Revenue		0	0	0	0	0	0	0	0	0	0	0	0	0	0
Revenue	7,015	0	0	0	0	0	0	0	50,000	144,936	108,199	178,217	436,897	628,877	868,063
Operating expenses:															
COGS	0	0	0	0	0	0	0	0	0	1,558	5,058	7,475	10,816	14,101	17,156
% of Product Sales	NM	NM	NM	NM	NM	NM	NM	NM	NM	10%	8%	7%	7%	7%	7%
Research and Development	11,551	11,434	3,525	5,920	6,763	7,000	23,208	36,000	50,000	62,000	68,200	75,020	90,024	108,029	129,635
% of Revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	43%	63%	42%	21%	17%	15%
SG&A	4,989	5,449	2,355	2,853	2,844	3,100	11,152	13,000	18,000	31,000	38,000	41,000	44,300	47,115	49,021
% of Revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	21%	35%	23%	10%	7%	6%
Total Operating Expenses	16,265	16,883	5,880	8,773	9,607	10,100	34,360	49,000	68,000	94,538	111,258	123,495	145,140	169,245	195,811
Income (Loss) from operations	(9,250)	(16,883)	(5,880)	(8,773)	(9,607)	(10,100)	(34,360)	(49,000)	(18,000)	50,438	(3,059)	54,722	291,757	459,632	672,253
Other income (expense):															
Preferred stock warrant remeasurement	489	0	0	0	0	0	0	0	0	0	0	0	0	1	2
Interest income	2	0	0	0	21	134	155	480	490	640	731	831	1,395	2,544	4,293
Loss on extinguishment of debt	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Interest expense	(1,342)	(1,140)	(153)	(177)	0	0	(330)	0	0	0	0	0	0	0	0
Total other income (expense)	(871)	(1,140)	(153)	(177)	21	134	(175)	480	490	640	731	831	1,395	2,545	4,295
Pre-tax income	(10,121)	(18,023)	(6,033)	(8,950)	(9,586)	(9,966)	(34,535)	(48,520)	(17,510)	51,077	(2,328)	55,554	293,152	462,177	676,548
Tax	0	0	0	0	0	0	0	0	0	18,889	0	20,555	108,466	171,006	250,323
<i>Tax rate</i>										37%	0%	37%	37%	37%	37%
Less: Accretion and dividends on redeemable convertible preferred stock	(4,097)	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Net loss	(14,218)	(18,023)	(6,033)	(8,950)	(9,586)	(9,966)	(34,535)	(48,520)	(17,510)	32,179	(2,328)	34,999	184,685	291,172	426,225
Non-GAAP EPS	\$ (516.00)	\$ (611.72)	\$ (0.56)	\$ (0.51)	\$ (0.54)	\$ (0.56)	\$ (2.16)	\$ (2.48)	\$ (0.78)	\$ 1.34	\$ (0.09)	\$ 1.38	\$ 7.03	\$ 10.69	\$ 15.07
Pro forma weighted average shares outstanding	28	29	10,822	17,685	17,707	17,757	15,993	19,553	22,378	24,003	24,678	25,428	26,278	27,228	28,280

Source: Company data, Jefferies Group LLC estimate
11/5/2014

DRNA: Historical and Projected Changes in Financial Position

December 31 Fiscal Year (\$000s, except per share)	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
CASH FLOWS FROM OPERATING ACTIVITIES:											
Net loss	(10,121)	(18,023)	(34,535)	(48,520)	(17,510)	32,179	(2,328)	34,999	184,685	291,172	426,225
Adjustments to reconcile net loss to net cash used in operating activities											
Deferred revenue	(1,014)										
Depreciation	551	523	575	632	696	765	842	926	1,019	1,120	1,232
Stock-based compensation		495									
Loss on extinguishment of debt	-	318									
Non-cash interest expense	-										
Amortization of debt discount	201	193									
Amortization of debt issuance costs	24	24									
Utilization of NOL Carryforwards			-	-	-	18,899	-	20,555	-	-	-
Decrease in fair value of preferred stock warrant	(469)	(126)									
Changes in operating assets and liabilities:											
Research and license receivable	(4,857)	5,018	-	-	-	-	-	-	-	-	-
Prepaid expenses and other current assets	28	57									
Accounts payable	(216)	116									
Accrued expenses and other current liabilities	49	1,016									
Deferred rent	(38)	(60)									
Net cash used in operating activities	(15,737)	(10,449)	(33,960)	(47,888)	(16,814)	51,843	(1,486)	56,479	185,704	292,292	427,458
CASH FLOWS FROM INVESTING ACTIVITIES—											
Purchases of property and equipment	(120)	(413)	(496)	(595)	(5,947)	(6,542)	(7,196)	(7,916)	(8,707)	(9,578)	(10,536)
Net cash used in investing activities	(120)	(413)	(496)	(595)	(5,947)	(6,542)	(7,196)	(7,916)	(8,707)	(9,578)	(10,536)
CASH FLOWS FROM FINANCING ACTIVITIES:											
Proceeds from issuance of common stock	7	46	92,955	38,775	37,224	-	-	-	-	-	-
Repurchase of restricted common stock	-	(5)									
Payments of deferred issuance costs	-	(1,399)									
Proceeds from issuance of redeemable convertible preferred stock	-	57,000									
Redeemable convertible preferred stock issuance costs	-	(220)									
Proceeds from long-term debt	-	-									
Proceeds from bridge loan financing	-	3,000									
Payments of long-term debt issuance costs	-	-									
Payments of long-term debt fees	(136)	-									
Repayments of long-term debt principal	(2,834)	(4,140)	(4,587)	(439)							
Net cash provided by (used in) financing activities	(2,963)	54,282	88,368	38,336	37,224	-	-	-	-	-	-
INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	(18,820)	43,420	53,912	(10,147)	14,463	45,301	(8,682)	48,564	176,997	282,714	416,922
CASH AND CASH EQUIVALENTS—Beginning of period	22,490	3,670	47,090	101,002	90,855	105,318	150,619	141,936	190,500	367,497	690,211
CASH AND CASH EQUIVALENTS—End of period	3,670	47,090	101,002	90,855	105,318	150,619	141,936	190,500	367,497	650,211	1,067,133

Source: Company data, Jefferies Group LLC estimate
November 6, 2014

DRNA: Historical Condensed Balance Sheets

	(<i>\$000s</i>)	9/30/2013	12/31/2013	3/31/2014	6/30/2014	9/30/2014
ASSETS						
CURRENT ASSETS:						
Cash and cash equivalents		54,712	46,595	133,792	77,732	31,815
Held-to-maturity investments					22,526	80,089
Research and license agreement receivable		-	-	-	-	
Prepaid expenses and other current assets		413	2,058	906	1,016	
Total current assets		55,125	48,653	134,698	101,274	111,904
NONCURRENT ASSETS:						
Property and equipment-net		683	877	911	1,473	
Held-to-maturity investments					20,026	
Restricted cash		264	264	264	1,380	
Other assets		-	-	-	-	
Total noncurrent assets		947	1,141	1,175	22,879	-
TOTAL ASSETS		56,072	49,794	135,873	124,153	116,082
LIABILITIES AND STOCKHOLDERS' EQUITY						
CURRENT LIABILITIES:						
Accounts payable		1,557	1,700	715	1,062	
Current portion of long-term debt		4,471	4,587	3,785	-	
Deferred revenue		-	-	-	-	
Deferred rent		14	105	96	88	
Accrued expenses and other current liabilities		518	1,286	1,947	2,808	
Total current liabilities		6,560	7,678	6,543	3,958	-
NONCURRENT LIABILITIES:						
Long-term debt-net of current portion		1,407	260	-	-	-
Preferred stock warrant liability		436	529	-	-	-
Deferred revenue-net of current portion		-	-	-	-	-
Deferred rent-net of current portion		-	-	-	-	-
Total noncurrent liabilities		1,843	789	-	-	-
TOTAL LIABILITIES		8,403	8,467	6,543	3,958	5,212
Total stockholders' equity (deficit)		47,669	41,327	129,330	120,195	110,820
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY		56,072	49,794	135,873	124,153	116,082

Source: Company data, Jefferies Group LLC, Inc. estimates
November 6, 2014

Company Description

Dicerna Pharmaceuticals is a Watertown, MA-based therapeutics company focused on developing RNA interference (RNAi) technologies targeting liver and cancer. Dicerna has partnered two oncology development programs with the global pharmaceutical company Kyowa Hakko Kirin Co., Ltd. (KHK) targeting KRAS and CKAP5. DRNA's unpartnered programs are DCR-PH1 for Type 1 primary hyperoxaluria, a rare orphan disease resulting in renal failure, and DCR-M1711 targeting MYC for solid tumors.

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Jefferies Franchise Picks

page 5 of 8

Thomas Wei, Equity Analyst, (212) 284-2326, twei@jefferies.com

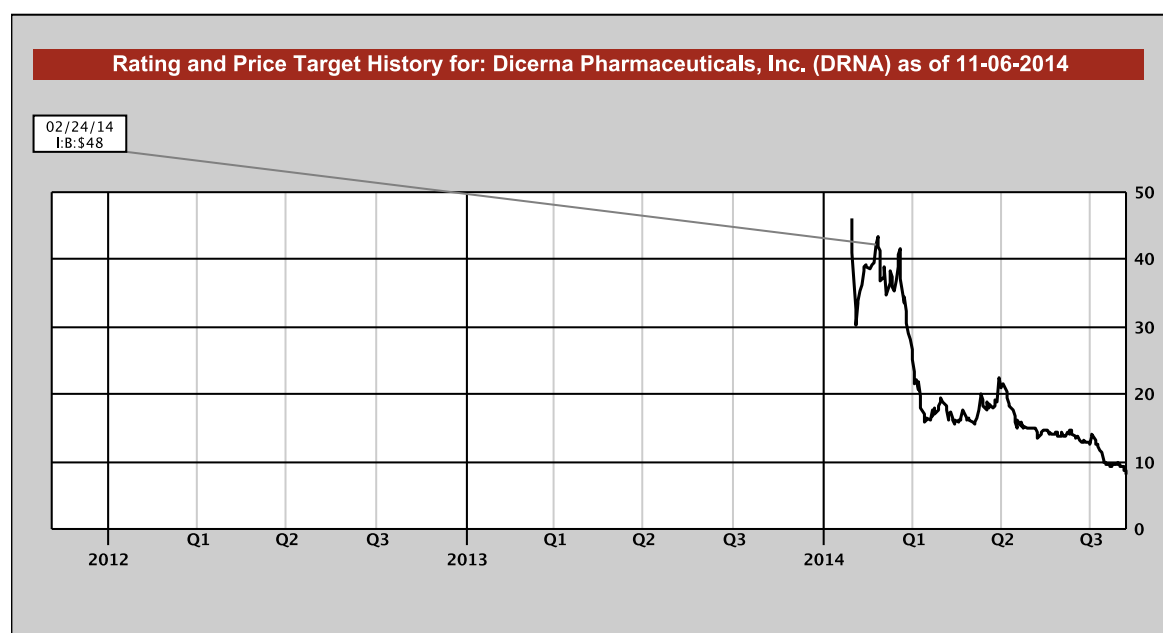
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Other Companies Mentioned in This Report

- Dicerna Pharmaceuticals, Inc. (DRNA: \$8.14, BUY)



Distribution of Ratings

Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY	1009	52.06%	264	26.16%
HOLD	790	40.76%	143	18.10%
UNDERPERFORM	139	7.17%	5	3.60%

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