

## COMPANY NOTE

Estimate Change

USA | Healthcare | Biotechnology

August 8, 2014

# Jefferies

## Dicerna Pharmaceuticals (DRNA) 2Q14 Update: Data Readouts Expected in 2015

### Key Takeaway

**We expect three Phase 1 trials to begin enrolling by 1H15, two for MYC and one for PH1. Key initial data for MYC and PH1 are expected in 2H15; a competitor program in PH1 should have results by year-end. We maintain our Buy rating and \$48 price target.**

**DCR-MYC Program On Track.** The first dose-finding Phase 1 trial in solid tumors, multiple myeloma, and lymphomas began enrollment in April. A first release of data is expected no later than 2H15. This trial will also include an expansion cohort treated at the maximum tolerated dose. Further, a second Phase 1 trial in advanced hepatocellular carcinoma (HCC) is expected to begin in Asia in 2H14. We view this program as high risk and assume a low probability of success in our valuation.

**DCR-PH1 Clinical Program Expected To Kick Off In 2015; Competitive Data At Year End.** 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. With the Phase 1 trial, we expect proof-of-concept results by 2H15. On the competitive landscape, Oxthera's Oxabact is expected to have data in December, using a 30% reduction at Week 8 as a responder endpoint. We continue to expect DCR-PH1 to be superior to Oxabact, which has had issues with bioavailability in the gut. On the call, DRNA noted that it expects DCR-PH1 to show benefit in all patients with PH1, including those with less severe disease. DRNA held an advisory board meeting with thought leaders who made it clear that reduction in oxalate levels would be beneficial for all genotypes. We note that PH1 patients with a specific genotype accounting for ~25% of the population respond well to Vitamin B6 therapy, which reduced oxalate levels by 70%+.

**Financials:** DRNA reiterated its expectation that it would have sufficient cash to fund operations through 2016. The company ended 2Q14 with \$120m in cash/equivalents.

### 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.	Prev.	Prev.
Rev. (MM)	--	0.0	--	0.0	--	--	--
<b>EPS</b>							
Mar	--	(0.56)A	--	--	--	--	--
Jun	(0.40)	(0.51)A	--	--	--	--	--
Sep	(0.42)	(0.53)	--	--	--	--	--
Dec	(0.44)	(0.56)	--	--	--	--	--
FY Dec	(1.77)	(2.15)	(2.38)	(2.48)	--	--	--
FY P/E		NM		NM			

Limited historical data due to recent company IPO

**BUY**

Price target \$48.00

Price \$14.35

### Financial Summary

Net Debt (MM):	(\$140.0)
Cash (MM):	\$140.0

### Market Data

52 Week Range:	\$46.00 - \$14.09
Total Entprs. Value (MM):	\$111.1
Market Cap. (MM):	\$251.1
Insider Ownership:	43.3%
Institutional Ownership:	38.3%
Shares Out. (MM):	17.5
Float (MM):	8.8
Avg. Daily Vol.:	168,331

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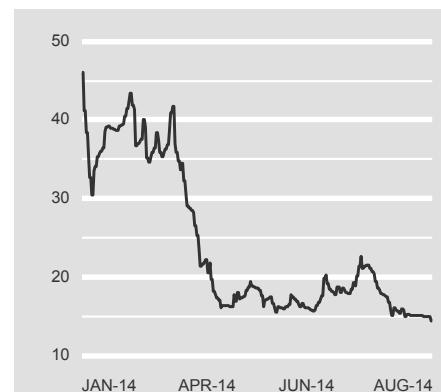
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### Price Performance



## DRNA: Historical and Projected Revenue and Earnings

December 31 Fiscal Year (\$000s, except per share)	2012	2013	1Q14A	2Q14A	3Q14E	4Q14E	2014	2015	2016	2017	2018	2019	2020	2021	2022
<u>End User Sales</u>															
U.S. PH1 Sales		0	0	0	0	0	0	0	0	15,581	63,225	106,783	154,514	201,448	245,079
Ex-U.S. PH1 Sales		0	0	0	0	0	0	0	0	22,073	89,895	155,040	224,326	293,117	355,972
WW PH1 Sales		0	0	0	0	0	0	0	0	37,654	153,120	261,823	378,840	494,565	601,051
WW DCR-M1711 Sales		0	0	0	0	0	0	0	0	0	0	0	66,150	667,229	1,829,961
<u>Dicerna Reported Revenue</u>															
U.S. PH1 Sales		0	0	0	0	0	0	0	0	15,581	63,225	106,783	154,514	201,448	245,079
Ex-U.S. PH1 Royalties		0	0	0	0	0	0	0	0	4,415	19,974	36,512	57,298	77,935	96,792
DCR-M1711 Royalties		0	0	0	0	0	0	0	0	0	0	9,922	100,084	274,494	451,193
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
<b>Revenue</b>	<b>7,015</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>50,000</b>	<b>144,996</b>	<b>108,199</b>	<b>178,217</b>	<b>436,897</b>	<b>628,877</b>	<b>868,063</b>
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	6,500	7,000	22,945	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	4,589	5,449	2,355	2,853	3,000	3,100	11,308	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%
<b>Total Operating Expenses</b>	<b>16,265</b>	<b>16,883</b>	<b>5,880</b>	<b>8,773</b>	<b>9,500</b>	<b>10,100</b>	<b>34,253</b>	<b>49,000</b>	<b>68,000</b>	<b>94,558</b>	<b>111,258</b>	<b>123,495</b>	<b>145,140</b>	<b>169,245</b>	<b>195,811</b>
<b>Income (Loss) from operations</b>	<b>(9,250)</b>	<b>(16,883)</b>	<b>(5,880)</b>	<b>(8,773)</b>	<b>(9,500)</b>	<b>(10,100)</b>	<b>(34,253)</b>	<b>(49,000)</b>	<b>(18,000)</b>	<b>50,438</b>	<b>(3,059)</b>	<b>54,722</b>	<b>291,757</b>	<b>459,632</b>	<b>672,253</b>
Other income (expense):															
Preferred stock warrant remeasurement	469	0	0	0	0	0	0	0	0	0	0	0	0	1	2
Interest income	2	0	0	0	144	132	277	503	559	731	823	923	1,487	2,637	4,386
Loss on extinguishment of debt		0	0	0	0	0	0	0	0	0	0	0	0	0	0
Interest expense	(1,342)	(1,140)	(153)	(177)	(69)	(69)	(469)	0	0	0	0	0	0	0	0
<b>Total other income (expense)</b>	<b>(871)</b>	<b>(1,140)</b>	<b>(153)</b>	<b>(177)</b>	<b>75</b>	<b>63</b>	<b>(192)</b>	<b>503</b>	<b>559</b>	<b>731</b>	<b>823</b>	<b>923</b>	<b>1,487</b>	<b>2,638</b>	<b>4,388</b>
<b>Pre-tax income</b>	<b>(10,121)</b>	<b>(18,023)</b>	<b>(6,033)</b>	<b>(8,950)</b>	<b>(9,425)</b>	<b>(10,037)</b>	<b>(34,445)</b>	<b>(48,497)</b>	<b>(17,441)</b>	<b>51,168</b>	<b>(2,237)</b>	<b>55,645</b>	<b>293,244</b>	<b>462,270</b>	<b>676,641</b>
Tax	0	0	0	0	0	0	0	0	0	18,932	0	20,589	108,500	171,040	250,357
Tax rate										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
<b>Net loss</b>	<b>(14,218)</b>	<b>(18,023)</b>	<b>(6,033)</b>	<b>(8,950)</b>	<b>(9,425)</b>	<b>(10,037)</b>	<b>(34,445)</b>	<b>(48,497)</b>	<b>(17,441)</b>	<b>32,236</b>	<b>(2,237)</b>	<b>35,057</b>	<b>184,744</b>	<b>291,230</b>	<b>426,284</b>
<b>Non-GAAP EPS</b>	<b>\$ (516.00)</b>	<b>\$ (611.72)</b>	<b>\$ (0.56)</b>	<b>\$ (0.51)</b>	<b>\$ (0.53)</b>	<b>\$ (0.56)</b>	<b>\$ (2.15)</b>	<b>\$ (2.48)</b>	<b>\$ (0.78)</b>	<b>\$ 1.34</b>	<b>\$ (0.09)</b>	<b>\$ 1.38</b>	<b>\$ 7.03</b>	<b>\$ 10.70</b>	<b>\$ 15.07</b>
Pro forma weighted average shares outstanding	28	29	10,822	17,685	17,735	17,785	16,007	19,553	22,378	24,003	24,678	25,428	26,278	27,228	28,280

Source: Company data, Jefferies Group LLC estimate  
8/8/2014

**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
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>											
Net loss	(10,121)	(18,023)	(34,445)	(48,497)	(17,441)	32,236	(2,237)	35,057	184,744	291,230	426,284
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			-	-	-	18,932	-	20,589	-	-	-
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)	(33,870)	(47,865)	(16,745)	51,934	(1,395)	56,571	185,762	292,350	427,516
<b>CASH FLOWS FROM INVESTING ACTIVITIES –</b>											
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)
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>											
Proceeds from issuance of common stock	7	46	92,955	47,940	46,022	-	-	-	-	-	-
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	47,501	46,022	-	-	-	-	-	-
<b>INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>(18,820)</b>	<b>43,420</b>	<b>54,002</b>	<b>(958)</b>	<b>23,330</b>	<b>45,392</b>	<b>(8,591)</b>	<b>48,656</b>	<b>177,055</b>	<b>282,772</b>	<b>416,980</b>
<b>CASH AND CASH EQUIVALENTS – Beginning of period</b>	<b>22,490</b>	<b>3,670</b>	<b>47,090</b>	<b>101,092</b>	<b>100,134</b>	<b>123,464</b>	<b>168,855</b>	<b>160,264</b>	<b>208,920</b>	<b>385,975</b>	<b>668,747</b>
<b>CASH AND CASH EQUIVALENTS – End of period</b>	<b>3,670</b>	<b>47,090</b>	<b>101,092</b>	<b>100,134</b>	<b>123,464</b>	<b>168,855</b>	<b>160,264</b>	<b>208,920</b>	<b>385,975</b>	<b>668,747</b>	<b>1,085,727</b>

Source: Company data, Jefferies Group LLC estimate  
August 8, 2014

**DRNA: Historical Condensed Balance Sheets**

(\$000s)	12/31/2011	12/31/2012	9/30/2013	12/31/2013	3/31/2014	6/30/2014
<b>ASSETS</b>						
<b>CURRENT ASSETS:</b>						
Cash and cash equivalents	22,490	3,670	54,712	46,595	133,792	77,732
Held-to-maturity investments						22,526
Research and license agreement receivable	161	5,018	-	-	-	-
Prepaid expenses and other current assets	372	344	413	2,058	906	1,016
<b>Total current assets</b>	<b>23,023</b>	<b>9,032</b>	<b>55,125</b>	<b>48,653</b>	<b>134,698</b>	<b>101,274</b>
<b>NONCURRENT ASSETS:</b>						
Property and equipment-net	1,314	883	683	877	911	1,473
Held-to-maturity investments						20,026
Restricted cash	264	264	264	264	264	1,380
Other assets	36	12	-	-	-	-
<b>Total noncurrent assets</b>	<b>1,614</b>	<b>1,159</b>	<b>947</b>	<b>1,141</b>	<b>1,175</b>	<b>22,879</b>
<b>TOTAL ASSETS</b>	<b>24,637</b>	<b>10,191</b>	<b>56,072</b>	<b>49,794</b>	<b>135,873</b>	<b>124,153</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>						
<b>CURRENT LIABILITIES:</b>						
Accounts payable	1,414	1,198	1,557	1,700	715	1,062
Current portion of long-term debt	2,834	4,140	4,471	4,587	3,785	-
Deferred revenue	830	-	-	-	-	-
Deferred rent	-	63	14	105	96	88
Accrued expenses and other current liabilities	221	270	518	1,286	1,947	2,808
<b>Total current liabilities</b>	<b>5,299</b>	<b>5,671</b>	<b>6,560</b>	<b>7,678</b>	<b>6,543</b>	<b>3,958</b>
<b>NONCURRENT LIABILITIES:</b>						
Long-term debt-net of current portion	8,735	4,660	1,407	260	-	-
Preferred stock warrant liability	800	331	436	529	-	-
Deferred revenue-net of current portion	184	-	-	-	-	-
Deferred rent-net of current portion	101	-	-	-	-	-
<b>Total noncurrent liabilities</b>	<b>9,820</b>	<b>4,991</b>	<b>1,843</b>	<b>789</b>	<b>-</b>	<b>-</b>
<b>TOTAL LIABILITIES</b>	<b>15,119</b>	<b>10,662</b>	<b>8,403</b>	<b>8,467</b>	<b>6,543</b>	<b>3,958</b>
<b>Total stockholders' equity (deficit)</b>	<b>9,518</b>	<b>(471)</b>	<b>47,669</b>	<b>41,327</b>	<b>129,330</b>	<b>120,195</b>
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<b>24,637</b>	<b>10,191</b>	<b>56,072</b>	<b>49,794</b>	<b>135,873</b>	<b>124,153</b>

Source: Company data, Jefferies Group LLC, Inc. estimates  
August 7, 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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The expected total return (price appreciation plus yield) for Buy rated stocks with an average stock price consistently below \$10 is 20% or more within a 12-month period as these companies are typically more volatile than the overall stock market. For Hold rated stocks with an average stock price consistently below \$10, the expected total return (price appreciation plus yield) is plus or minus 20% within a 12-month period. For Underperform rated stocks with an average stock price consistently below \$10, the expected total return (price appreciation plus yield) is minus 20% within a 12-month period.

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### Jefferies Franchise Picks

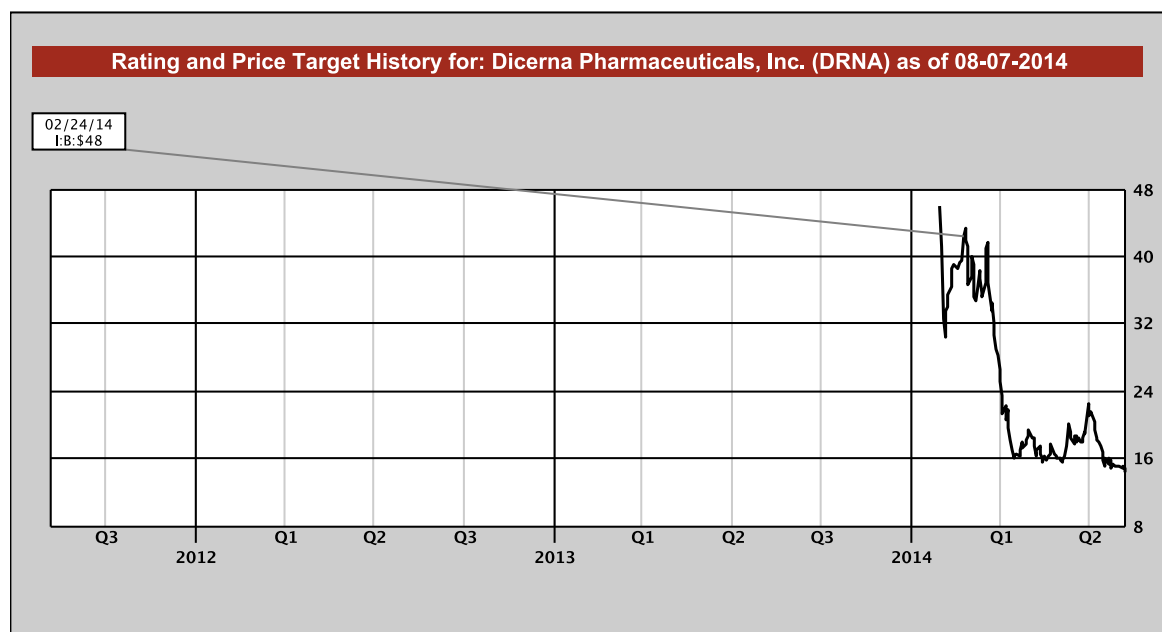
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## Distribution of Ratings

Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY	968	51.63%	252	26.03%
HOLD	758	40.43%	122	16.09%
UNDERPERFORM	149	7.95%	8	5.37%

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