

COMPANY NOTE

Estimate Change

USA | Healthcare | Biotechnology

May 14, 2014

Jefferies

Dicerna Pharmaceuticals (DRNA) 1Q14 DRNA Earnings: Data Readouts Expected in 2015

Key Takeaway

The next major catalyst for DRNA will be an update from meeting with the FDA on initiation of a DCR-PH1 Phase 1 trial. We believe that, even if the FDA requires a healthy volunteer study first, DRNA is looking at ways in which this study may still be able to validate the drug's mechanism of action. We continue to expect data from this trial and the DCR-MYC Phase 1 trial in 2H15. We maintain our Buy and \$48 price target.

DCR-MYC Phase 1 Trial Began This Quarter. The dose escalation portion for the first DCR-MYC (formerly DCR-M1711) trial in solid tumors, multiple myeloma and lymphoma began this quarter to determine the maximum tolerated dose (MTD) in non-hepatocellular carcinoma (HCC) patients, followed by an expansion cohort at the MTD. A second Phase 1 trial in advanced HCC (likely Nexavar intolerant or failures) is expected to begin in 2H14 and will follow the same MTD and then expansion format. We expect proof-of-concept data in 2H15. DRNA does not believe there will be significant adverse events associated with maximal MYC knockdown, as knockout mice only experienced minor myelosuppression issues. However, with a lesser degree of knockdown than the 70% seen in preclinical models, DRNA believes that DCR-MYC will still be efficacious, as 25% preclinical knockdown was sufficient for an anti-tumor effect.

DCR-PH1 On Track For Phase 1 Initiation In Early 2015. DRNA will select a clinical candidate this year and initiate the Phase 1 trial in Type 1 primary hyperoxaluria (PH1) in early 2015. The company has an upcoming meeting with the FDA to discuss the program and it is still unclear whether the first trial would be in healthy volunteers or PH1 patients. Either a healthy volunteer or patient trial could have efficacy endpoints that look at urinary oxalate excretion, as well as surrogate markers that could be early predictors of efficacy. Even with a healthy volunteer study, we would expect validation of the platform, as volunteers could potentially be fed diets that cause oxalate excretion.

Next Target Expected This Year. DRNA expects to reveal its third drug target this calendar year. The major criteria in selecting targets are: a high likelihood of clinical success and the potential to retain as much of the economics as possible. Thus, we would assume DRNA's priority will continue to be on orphan opportunities like PH1.

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	--	--	--
EPS							
Mar	(0.39)	(0.56)A	--	--	--	--	--
Jun	--	(0.40)	--	--	--	--	--
Sep	(0.43)	(0.42)	--	--	--	--	--
Dec	(0.45)	(0.44)	--	--	--	--	--
FY Dec	(1.67)	(1.77)	(2.37)	(2.38)	--	--	--
FY P/E		NM		NM			

Limited historical data due to recent company IPO

BUY

Price target \$48.00

Price \$16.60

Financial Summary

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

Market Data

52 Week Range:	\$46.00 - \$15.00
Total Entprs. Value (MM):	\$150.5
Market Cap. (MM):	\$290.5
Insider Ownership:	43.3%
Institutional Ownership:	38.3%
Shares Out. (MM):	17.5
Float (MM):	4.8
Avg. Daily Vol.:	183,325

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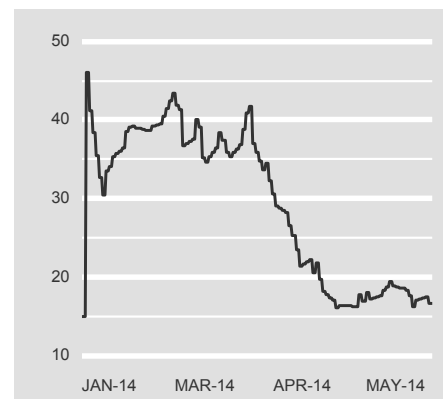
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Price Performance



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Financials And Guidance. DRNA reported a net cash loss in 1Q14 of \$6m and ended the quarter with \$134m in cash. The company continues to expect the current cash balance to be sufficient to fund operations through 2016.

DRNA: Historical and Projected Revenue and Earnings

December 31 Fiscal Year (\$000s, except per share)	2012	2013	1Q14A	2Q14E	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
Revenue	7,015	0	0	0	0	0	0	0	50,000	144,996	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	4,750	5,250	5,500	19,025	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	2,400	2,400	2,400	9,555	11,000	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	7,150	7,650	7,900	28,580	47,000	68,000	94,558	111,258	123,495	145,140	169,245	195,811
Income (Loss) from operations	(9,250)	(16,883)	(5,880)	(7,150)	(7,650)	(7,900)	(28,580)	(47,000)	(18,000)	50,438	(3,059)	54,722	291,757	459,632	672,253
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	163	154	144	461	538	599	771	863	964	1,528	2,678	4,427
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)	(69)	(69)	(69)	(361)	0	0	0	0	0	0	0	0
Total other income (expense)	(871)	(1,140)	(153)	93	84	75	100	538	599	771	863	964	1,528	2,679	4,429
Pre-tax income	(10,121)	(18,023)	(6,033)	(7,057)	(7,566)	(7,825)	(28,480)	(46,462)	(17,401)	51,209	(2,196)	55,686	293,285	462,311	676,682
Tax	0	0	0	0	0	0	0	0	0	18,947	0	20,604	108,515	171,055	250,372
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
Net loss	(14,218)	(18,023)	(6,033)	(7,057)	(7,566)	(7,825)	(28,480)	(46,462)	(17,401)	32,261	(2,196)	35,082	184,769	291,256	426,310
Non-GAAP EPS	\$ (516.00)	\$ (611.72)	\$ (0.56)	\$ (0.40)	\$ (0.42)	\$ (0.44)	\$ (1.77)	\$ (2.38)	\$ (0.78)	\$ 1.34	\$ (0.09)	\$ 1.38	\$ 7.03	\$ 10.70	\$ 15.07
Pro forma weighted average shares outstanding	28	29	10,822	17,763	17,813	17,863	16,065	19,553	22,378	24,003	24,678	25,428	26,278	27,228	28,280

Source: Company data, Jefferies Group LLC estimate
5/13/2014

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)	(28,480)	(46,462)	(17,401)	32,261	(2,196)	35,082	184,769	291,256	426,310
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			-	-	-	18,947	-	20,604	-	-	-
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)	(27,905)	(45,830)	(16,705)	51,974	(1,354)	56,612	185,788	292,376	427,542
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	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	-	-	-	-	-	-
INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	(18,820)	43,420	59,967	1,077	23,370	45,432	(8,550)	48,696	177,081	282,798	417,006
CASH AND CASH EQUIVALENTS – Beginning of period	22,490	3,670	47,090	107,057	108,133	131,503	176,935	168,385	217,081	394,162	676,960
CASH AND CASH EQUIVALENTS – End of period	3,670	47,090	107,057	108,133	131,503	176,935	168,385	217,081	394,162	676,960	1,093,966

Source: Company data, Jefferies Group LLC estimate
May 13, 2014

DRNA: Historical Condensed Balance Sheets

	(<i>\$000s</i>)	12/31/2011	12/31/2012	9/30/2013	12/31/2013	3/31/2014
ASSETS						
CURRENT ASSETS:						
Cash and cash equivalents		22,490	3,670	54,712	46,595	133,792
Research and license agreement receivable		161	5,018	-	-	-
Prepaid expenses and other current assets		372	344	413	2,058	906
Total current assets		23,023	9,032	55,125	48,653	134,698
NONCURRENT ASSETS:						
Property and equipment-net		1,314	883	683	877	911
Restricted cash		264	264	264	264	264
Other assets		36	12	-	-	-
Total noncurrent assets		1,614	1,159	947	1,141	1,175
TOTAL ASSETS		24,637	10,191	56,072	49,794	135,873
LIABILITIES AND STOCKHOLDERS' EQUITY						
CURRENT LIABILITIES:						
Accounts payable		1,414	1,198	1,557	1,700	715
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
Accrued expenses and other current liabilities		221	270	518	1,286	1,947
Total current liabilities		5,299	5,671	6,560	7,678	6,543
NONCURRENT LIABILITIES:						
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	-	-	-	-
Total noncurrent liabilities		9,820	4,991	1,843	789	-
TOTAL LIABILITIES		15,119	10,662	8,403	8,467	6,543
Total stockholders' equity (deficit)		9,518	(471)	47,669	41,327	129,330
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY		24,637	10,191	56,072	49,794	135,873

Source: Company data, Jefferies Group LLC, Inc. estimates
May 13, 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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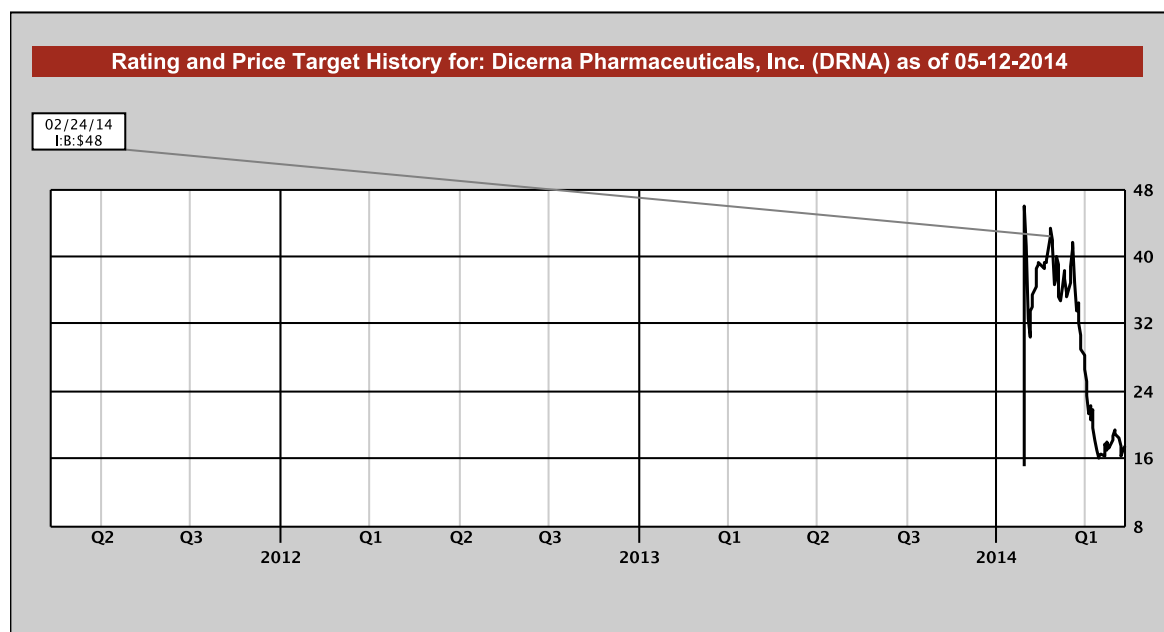
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			Count	Percent
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