

COMPANY NOTE

Estimate Change

USA | Healthcare | Biotechnology

March 27, 2014

Jefferies

Dicerna Pharmaceuticals (DRNA) 4Q13 DRNA Update: Multiple Milestones In 2014

Key Takeaway

The next key catalyst for DRNA is the initiation of two Phase 1 trials of DCR-M1711, which should begin in 2Q14. We expect topline Phase 1 results in 2015. Phase 1 studies for primary hyperoxaluria will start in 1H15. DRNA will disclose a new therapeutic area of development later in 2014. We expect critical human validation of both its technology and lead programs in 2H15.

DCR-M1711 Phase 1 Study To Start In 2Q14. DRNA plans to initiate the first of two Phase 1 studies of DCR-M1711 in 2Q14. The first study will enroll patients with non-hepatic cellular carcinoma (HCC) tumors and will be a standard dose escalation trial to determine the maximum tolerated dose (MTD) in non-HCC patients followed by an expansion cohort at the MTD. The second Phase 1 study will be in patients with advanced HCC and is expected to begin in 2H14 and will have two parts as well, MDT and an expansion cohort.

DRNA's RNAi Platform Should Be Validated With Multiple Data Readouts In 2015. DRNA's lead unpartnered candidates are DCR-PH1 for Type 1 primary hyperoxaluria (PH1) and DCR-M1711 targeting MYC for solid tumors. DRNA plans to complete the optimization of DCR-PH1 and declare a clinical candidate in 2Q14, followed by a Phase 1 study in PH1 in early 2015 with data in 2H15. DRNA plans to expand its RNAi platform into other liver-targeted orphan diseases, and we expect the company to disclose a second orphan liver candidate in 2H14, with a Phase 1 study to start in 2H15. We believe positive early preclinical data for DCR-PH1 will provide safety and mechanistic validation for the platform and support DRNA's use of it for other liver-targeted orphan diseases to rapidly build a robust pipeline. Proof-of-concept data should be available in 2H15 for both DCR-PH1 and DCR-M1711.

Financials and Guidance. DRNA reported a net loss in 4Q13 of \$19m. DRNA ended the year with cash and cash equivalents of \$47m and raised net IPO proceeds of \$93m in February 2014, for a pro forma cash balance of \$140m. The company expects the current cash balance will be sufficient to fund operations through 2016. DRNA does not expect any milestone revenues in 2014, and guided to an increase in both G&A and R&D in 2014 versus 2013.

Valuation/Risks

We derive our \$48 price target on a DCF based sum-of-parts valuation (\$16 PH1 + \$11 MYC + \$15 Pipeline + \$5 cash). Risks include: clinical, regulatory, and commercial.

USD	Prev.	2012A	Prev.	2013A	Prev.	2014E	Prev.	2015E
Rev. (MM)	--	7.0	--	0.0	--	0.0	--	0.0
EV/Rev		56.5x						
EPS								
Mar	--	--	--	(1.55)	(0.40)	(0.39)	--	--
Jun	--	--	--	(1.17)	--	(0.40)	--	--
Sep	--	--	--	(0.94)	(0.40)	(0.43)	--	--
Dec	--	--	(1.08)	(1.11)	(0.40)	(0.45)	--	--
FY Dec	--	(4.95)	(4.55)	(4.60)	(1.60)	(1.67)	--	(2.37)
FY P/E		NM		NM		NM		NM

BUY

Price target \$48.00

Price \$30.61

Financial Summary

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

Market Data

52 Week Range:	\$46.00 - \$15.00
Total Entprs. Value (MM):	\$395.7
Market Cap. (MM):	\$535.7
Insider Ownership:	43.3%
Institutional Ownership:	38.3%
Shares Out. (MM):	17.5
Float (MM):	4.0
Avg. Daily Vol.:	NA

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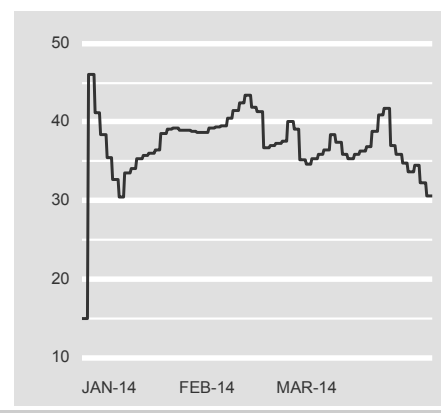
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Price Performance



DRNA: Historical and Projected Revenue and Earnings

December 31 Fiscal Year (\$000s, except per share)	2012	1Q13A	2Q13A	3Q13A	4Q13A	2013	1Q14E	2Q14E	3Q14E	4Q14E	2014	2015	2016	2017	2018	2019	2020	2021	2022
End User Sales																			
U.S. PH1 Sales		0	0	0	0	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	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	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	0	0	0	0	66,150	667,229	1,829,961
Dicerna Reported Revenue																			
U.S. PH1 Sales		0	0	0	0	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	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	0	0	0	0	9,922	100,084	274,494	451,193
Milestones		0	0	0	0	0	0	0	0	0	0	0	0	50,000	125,000	25,000	25,000	75,000	75,000
Other Revenue		0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Revenue	7,015	0	0	0	0	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	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	NM	NM	NM	NM	10%	8%	7%	7%	7%	7%
Research and Development	11,551	2,455	2,455	2,455	4,194	11,558	4,500	4,750	5,250	5,500	20,000	36,000	51,000	62,000	68,200	75,020	90,024	108,029	129,635
% of Revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	43%	43%	42%	43%	27%	25%
SG&A	4,589	1,192	1,192	1,192	2,243	5,820	2,250	2,300	2,350	2,400	9,300	11,000	18,000	33,000	38,000	41,000	44,300	47,115	49,021
% of Revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	23%	35%	23%	10%	7%	6%
Total Operating Expenses	16,265	3,647	3,647	3,647	6,437	17,378	6,750	7,050	7,600	7,900	29,300	47,000	69,000	96,558	111,258	123,495	145,140	169,245	195,811
Income (Loss) from operations	(9,250)	(3,647)	(3,647)	(3,647)	(6,437)	(17,378)	(6,750)	(7,050)	(7,600)	(7,900)	(29,300)	(47,000)	(19,000)	48,438	(3,059)	54,722	291,757	459,632	672,253
Other income (expense):																			
Preferred stock warrant remeasurement	469	73	73	73	0	219	0	0	0	0	0	0	0	0	0	0	0	1	2
Interest income	2	0	0	0	(282)	(281)	58	50	41	31	180	658	967	1,255	1,345	1,448	2,014	3,165	4,916
Loss on extinguishment of debt		(106)	(106)	(106)	0	(318)	0	0	0	0	0	0	0	0	0	0	0	0	0
Interest expense	(1,342)	(253)	(253)	(253)	0	(760)	(69)	(69)	(69)	(69)	(277)	0	0	0	0	0	0	0	0
Total other income (expense)	(871)	(286)	(286)	(286)	(282)	(1,140)	(11)	(20)	(28)	(38)	(97)	658	967	1,255	1,345	1,448	2,014	3,166	4,918
Pre-tax income	(10,121)	(3,933)	(3,933)	(3,933)	(6,719)	(18,518)	(6,761)	(7,070)	(7,628)	(7,938)	(29,397)	(46,342)	(18,033)	49,693	(1,715)	56,170	293,771	462,798	677,171
Tax	0	0	0	0	0	0	0	0	0	0	0	0	0	18,386	0	20,783	108,695	171,235	250,553
<i>Tax rate</i>															0%	37%	37%	37%	37%
Less: Accretion and dividends on redeemable convertible preferred stock	(4,097)	(793)	(793)	(793)	(9)	(2,388)	0	0	0	0	0	0	0	0	0	0	0	0	0
Net loss	(14,218)	(4,726)	(4,726)	(4,726)	(6,728)	(20,906)	(6,761)	(7,070)	(7,628)	(7,938)	(29,397)	(46,342)	(18,033)	31,306	(1,715)	35,387	185,075	291,563	426,618
Non-GAAP EPS	\$ (4.95)	\$ (1.55)	\$ (1.17)	\$ (0.94)	\$ (1.11)	\$ (4.60)	\$ (0.39)	\$ (0.40)	\$ (0.43)	\$ (0.45)	\$ (1.67)	\$ (2.37)	\$ (0.81)	\$ 1.30	\$ (0.07)	\$ 1.39	\$ 7.04	\$ 10.71	\$ 15.09
Pro forma weighted average shares outstanding	2,046	3,046	4,046	5,046	6,046	4,546	17,528	17,578	17,628	17,678	17,603	19,553	22,378	24,003	24,678	25,428	26,278	27,228	28,280

Source: Company data, Jefferies Group LLC estimate
3/27/2014

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
CASH FLOWS FROM OPERATING ACTIVITIES:									
Net loss	(10,121)	(18,518)	(29,397)	(46,342)	(18,033)	31,306	(1,715)	35,387	185,075
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
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			-	-	-	18,386	-	20,783	-
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)	(10,944)	(28,822)	(45,709)	(17,338)	50,458	(873)	57,096	186,094
CASH FLOWS FROM INVESTING ACTIVITIES –									
Purchases of property and equipment	(120)	(413)	(496)	(595)	(5,947)	(6,542)	(7,196)	(7,916)	(8,707)
Net cash used in investing activities	(120)	(413)	(496)	(595)	(5,947)	(6,542)	(7,196)	(7,916)	(8,707)
CASH FLOWS FROM FINANCING ACTIVITIES:									
Proceeds from issuance of common stock	7	46	92,955	98,700	94,752	-	-	-	-
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	98,261	94,752	-	-	-	-
INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	(18,820)	42,925	59,050	51,957	71,467	43,916	(8,069)	49,180	177,387
CASH AND CASH EQUIVALENTS—Beginning of period	22,490	3,670	46,595	105,645	157,602	229,069	272,985	264,916	314,096
CASH AND CASH EQUIVALENTS—End of period	3,670	46,595	105,645	157,602	229,069	272,985	264,916	314,096	491,483

Source: Company data, Jefferies Group LLC estimate
March 27, 2014

DRNA: Historical Condensed Balance Sheets

	(<i>\$000s</i>)	12/31/2011	12/31/2012	9/30/2013	12/31/2013
ASSETS					
CURRENT ASSETS:					
Cash and cash equivalents		22,490	3,670	54,712	46,595
Research and license agreement receivable		161	5,018	-	-
Prepaid expenses and other current assets		372	344	413	2,058
Total current assets		23,023	9,032	55,125	48,653
NONCURRENT ASSETS:					
Property and equipment-net		1,314	883	683	877
Restricted cash		264	264	264	264
Other assets		36	12	-	-
Total noncurrent assets		1,614	1,159	947	1,141
TOTAL ASSETS		24,637	10,191	56,072	49,794
LIABILITIES AND STOCKHOLDERS' EQUITY					
CURRENT LIABILITIES:					
Accounts payable		1,414	1,198	1,557	1,700
Current portion of long-term debt		2,834	4,140	4,471	4,587
Deferred revenue		830	-	-	-
Deferred rent		-	63	14	105
Accrued expenses and other current liabilities		221	270	518	1,286
Total current liabilities		5,299	5,671	6,560	7,678
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
Total stockholders' equity (deficit)		9,518	(471)	47,669	41,327
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY		24,637	10,191	56,072	49,794

Source: Company data, Jefferies Group LLC, Inc. estimates
March 27, 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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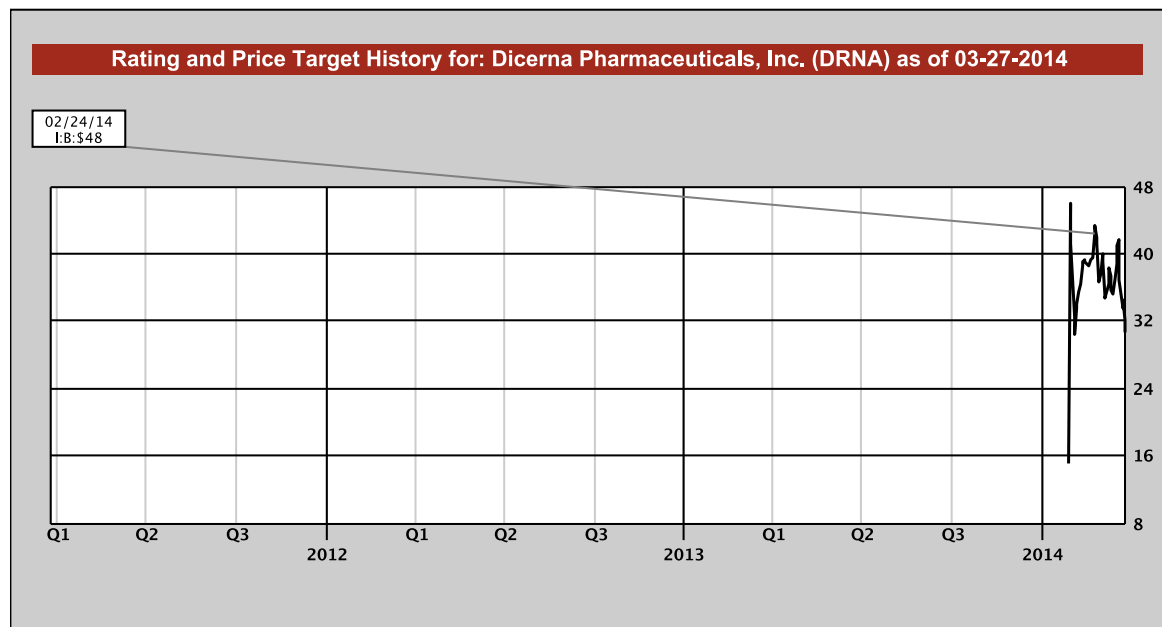
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Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
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