

Reason for report:

EARNINGS

ONCONOVA THERAPEUTICS, INC.

Phase III in Lower-Risk MDS in 2H:14 After Regulatory Discussions

• **Bottom Line:** ONTX reported 1Q:14 earnings and provided incremental updates on its pipeline, including announcing the initiation of a Phase I trial of oral rigosertib in combination with cisplatin and radiotherapy in patients with head and neck cancer. The company continues to anticipate holding discussions with the FDA and EMA in 2Q and 3Q of 2014 regarding the development of IV rigosertib in higher-risk myelodysplastic syndrome (MDS). Dialogue with these authorities is ongoing regarding the design of a Phase III trial of oral rigosertib in lower-risk MDS. In front-line MDS patients, oral rigosertib is in the dose escalation stage of a Phase I/II study in combination with azacitidine, and the Phase II portion is expected to commence in 2H:14. Given the failure of IV rigosertib to hit the primary endpoint in the Phase III ONTIME trial in high-risk MDS, we continue to focus on the lower-risk MDS opportunity.

• **Assessment of the prognostic genomic methylation marker for low-risk MDS continues.** ONTX expects to initiate a Phase III trial of oral rigosertib in 2H:14 following an analysis of this potential biomarker in 20 patients (currently enrolling). Management stated that based on the results from this cohort, the Phase III trial could be designed to either stratify or select patients based on methylation profile.

• **1Q:14 Review and Model Update:** Total 4Q:13 revenue was \$0.4M, below our and consensus estimates of \$1.9M and \$1.2M, respectively. Total 1Q operating expenses were \$19.2M, higher than our estimates of \$18.3M, driven by higher R&D spend. ONTX ended 1Q with \$84.6M in cash compared to \$100M at the end of 2013 and indicated it would have enough on hand to continue through mid-2015. 1Q EPS was (\$0.87) versus our estimate of (\$0.76) and consensus of (\$0.74). We have updated our model to account for these changes. Our price target remains at \$14.

Key Stats:

(NASDAQ:ONTX)

S&P 600 Health Care Index:	1,237.84
Price:	\$5.32
Price Target:	\$14.00
Methodology:	DCF analysis
52 Week High:	\$31.13
52 Week Low:	\$5.25
Shares Outstanding (mil):	21.6
Market Capitalization (mil):	\$114.9
Book Value/Share:	\$4.49
Cash Per Share:	\$3.92
Dividend (ann):	\$0.00
Dividend Yield:	0.0%



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	\$1.1	\$0.6	\$1.1	\$1.9	\$4.8	(\$1.03)	(\$0.64)	(\$0.97)	(\$0.68)	(\$6.12)	NM
2014E - New	\$0.4A	\$1.0	\$25.0	0.0	\$26.5	(\$0.87)A	(\$0.90)	\$0.17	(\$0.97)	(\$2.55)	NM
2014E - Old	\$1.9	\$1.0	\$25.0	0.0	\$27.9	(\$0.76)	(\$0.82)	\$0.24	(\$0.88)	(\$2.18)	NM
2015E - New	--	--	--	--	\$25.0	--	--	--	--	(\$1.95)	NM
2015E - Old	--	--	--	--	\$25.0	--	--	--	--	(\$1.69)	NM

Source: Company Information and Leerink Partners LLC Research
Revenues in millions.
EPS are GAAP.

INVESTMENT THESIS

ONTX is a late-stage story, with a Phase III trial in low-risk myelodysplastic syndrome (MDS) expected to begin in 2014. Although the IV formulation of rigosertib failed to show a statistically significant overall survival benefit in its Phase III ONTIME trial in higher-risk MDS, the numerical trend toward improvement in survival in the overall trial, along with a significant survival benefit in a post-hoc analysis of hypomethylating-agent refractory patients, suggests that the drug does have some activity. While we do not expect the company to attempt another trial in the higher-risk population, we believe oral rigosertib's opportunity in lower-risk MDS should be viewed independent of the outcome of ONTIME. In contrast to the higher-risk setting, more robust hematological responses such as transfusion independence were seen. MEDACorp key opinion leaders view rigosertib data in lower-risk patients to be stronger than in higher-risk MDS. ONTX is one of a minority of biotech companies that have been able to maintain full US rights to their lead compound near the finish line.

Onconova Expected Events

Event	Time	Comment
Rigosertib		
1st line, lower risk MDS (oral)	2014	In discussions with FDA on Phase III design, seeking SPA; Second Phase II trial in patients not receiving ESAs ongoing; enrolling 20 patients to prognostic genomic methylation marker
2nd-line Higher-risk MDS (IV)	2014	Seeking Type A meeting with FDA to discuss next steps in advancing IV rigosertib in higher-risk MDS
1st line, lower risk MDS (oral)	2H:14	Initiation of Phase III trial design following FDA meeting regarding special protocol (SPA)
1st line MDS in combo with azacitidine	2H:14	Initiation of Phase II dosing

Source: Company reports

Onconova Pipeline

Agent	Phase	Status/Anticipated Milestones
Rigosertib Single-agent		
2nd-line Higher-risk MDS (IV)	III	Phase III ONTIME trial failed to meet primary endpoint; discussion with FDA and EMA on path forward are ongoing
1st-line Lower-risk MDS (oral)	II	In discussions with FDA on Phase III design seeking SPA, enrolling a cohort of 20 patients to explore a prognostic genomic methylation marker; Second Phase II trial in patients not receiving ESAs ongoing
Head and Neck Cancer (oral)	II	Complete Phase II enrollment 2H:14
Rigosertib in Combination		
1st-line MDS (oral) Vidaza Combination	I/II	Initiated 3Q:13; in dose escalation stage
Head and Neck Cancer (oral) combination with cisplatin and radiotherapy	I	Initiated Phase I
ON 013105		
Lymphomas and ALL (IV)	I	On-going Phase I Trial
Recilisib		
Acute Radiation Syndromes (SC and oral)	I	Seeking Government Funding

Source: Company reports

VALUATION

Our 12-month price target on ONTX is \$14 based on DCF methodology. We assume rigosertib launches in lower-risk MDS in 2017E and no further development in higher-risk MDS. Our royalty assumption is 12-19% for ex-US sales. Our projection for probability-weighted (50% for lower-risk MDS) sales of rigosertib reaches \$241M for US, and ex-US royalties are projected to reach \$55M by 2029. We use a discount rate of 10%, which we believe is appropriate given the probability-weighted sales projection.

RISKS TO VALUATION

Risks to our valuation include the following:

- Binary clinical risk with Phase III readout of rigosertib in lower-risk MDS.
- Commercial and execution risks as a small company.
- Financing risk – ONTX ended 1Q14 with \$84.6M in cash, which together with anticipated milestone payments we estimate to be sufficient to fund operations into 2015, and the company may have additional financing needs before turning cash flow positive.

ONTX Income Statement (\$000)	2011A	2012A	2013A	Mar-14E	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E
Collaboration agreements/Milestones						25,000		25,000	25,000	50,000	50,000												
Royalties									0	0	2,411	9,234	17,295	26,414	28,777	31,312	34,029	36,942	40,061	43,400	46,973	50,794	54,878
Sales									0	0	37,098	97,719	123,533	151,804	159,871	168,342	177,236	186,574	196,376	206,666	217,465	228,800	240,695
Total revenue	1,487	46,190	4,753	447	1,000	25,000	0	26,447	25,000	50,000	89,509	106,954	140,827	178,218	188,648	199,654	211,266	223,515	236,437	250,065	264,438	279,594	295,574
COGS										4,000	7,161	7,818	9,883	12,144	12,790	13,467	14,179	14,926	15,710	16,533	17,397	18,304	19,256
% of revenue										8%	8%	8%	8%	8%	8%	8%	8%	8%	8%	8%	8%	8%	8%
R&D	22,624	52,762	50,182	14,248	14,533	14,824	15,120	58,725	63,504	65,410	67,372	69,393	71,475	73,619	75,828	78,102	80,445	82,859	85,345	87,905	90,542	93,258	96,056
SG&A	6,436	15,707	16,793	4,932	6,000	6,000	6,000	24,000	24,000	34,000	54,000	56,700	59,535	62,512	65,637	68,919	72,365	74,536	76,772	79,075	81,448	83,891	86,408
Total operating expenses	29,060	68,469	66,975	19,180	20,533	20,824	21,120	81,657	87,504	103,410	128,533	133,910	140,892	148,275	154,255	160,489	166,990	172,321	177,827	183,514	189,387	195,453	201,719
Net income (loss) from operations	(27,573)	(22,279)	(62,222)	(18,733)	(19,533)	4,176	(21,120)	(55,210)	(62,504)	(53,410)	(39,023)	(26,957)	(65)										
Change in fair value of warrant liability	1,287	367	42	16																			
Interest expense	(19)	(8,608)	(4)																				
Other income, net	11	608	63	1	0	0	0	1	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Net income (loss) before income taxes	(26,294)	(29,912)	(62,121)	(18,716)	(19,533)	4,176	(21,120)	(55,209)	(62,504)	(53,410)	(39,023)	(26,957)	(65)	29,943	34,393	39,165	44,276	51,195	58,610	66,552	75,051	84,140	93,854
Provision (benefit) for income taxes	0	0	435	0	0	0	0	0	0					10,480	12,038	13,708	15,497	17,918	20,514	23,293	26,268	29,449	32,849
Tax rate														35%	35%	35%	35%	35%	35%	35%	35%	35%	35%
Net loss attributable to non-controlling interest				37																			
Net income (loss)	(26,294)	(29,912)	(62,556)	(18,679)	(19,533)	4,176	(21,120)	(55,209)	(62,504)	(53,410)	(39,023)	(26,957)	(65)	19,463	22,356	25,457	28,779	33,276	38,097	43,259	48,783	54,691	61,005
Accretion of preferred stock	(4,020)	(3,953)	(2,320)	0	0	0	0	0	0														
Net income (loss) to common stockholders	(30,314)	(33,865)	(64,876)	(18,679)	(19,533)	4,176	(21,120)	(55,209)	(62,504)	(53,410)	(39,023)	(26,957)	(65)	19,463	22,356	25,457	28,779	33,276	38,097	43,259	48,783	54,691	61,005
Net loss per share	(14.18)	(2.67)	(6.12)	(0.87)	(0.90)	0.17	(0.97)	(2.55)	(1.95)	(1.63)	(1.02)	(0.69)	(0.00)	0.39	0.42	0.46	0.49	0.54	0.59	0.64	0.69	0.73	0.78
Basic shares	2,137	12,669	10,594	21,568	21,611	21,655	21,698	21,633	32,066	32,707	38,361	39,128	39,911	40,709	41,523	42,354	43,201	44,065	44,946	45,845	46,762	47,697	48,651
Dilutive shares				24,697	24,722	24,746	24,771	24,734	34,981	36,730	43,567	45,745	48,032	50,434	52,956	55,604	58,384	61,303	64,368	67,586	70,966	74,514	78,240

Source: Company Reports and Leerink Partners

Disclosures Appendix

Analyst Certification

I, Howard Liang, 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.

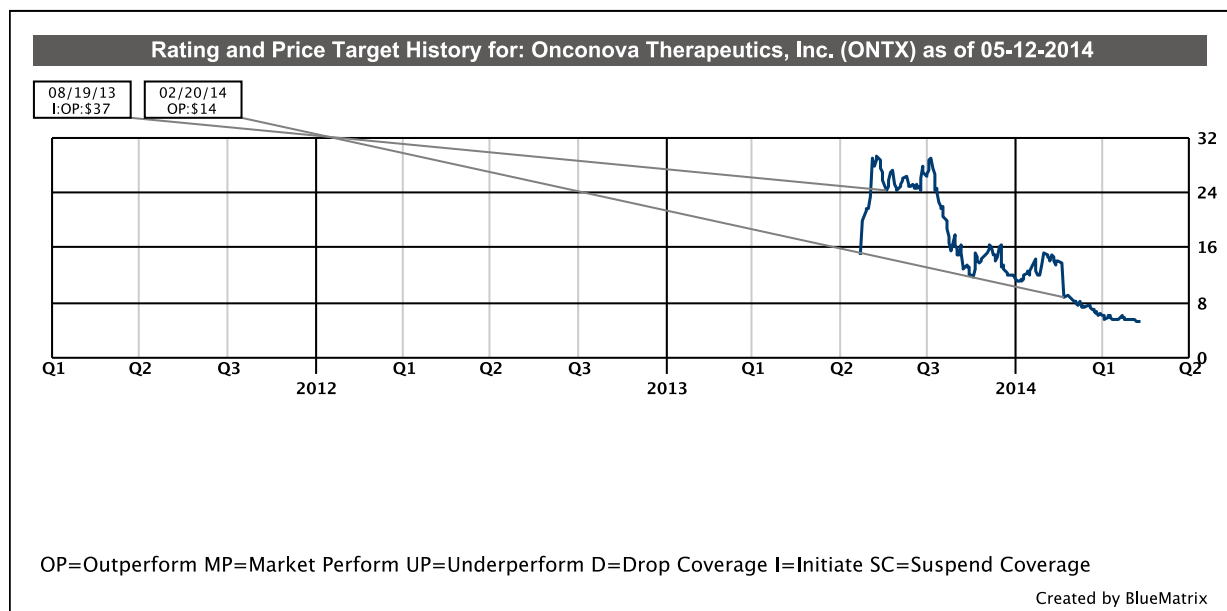
Valuation

Our 12-month price target on ONTX is \$14 based on DCF methodology. We assume rigosertib launches in lower-risk MDS in 2017E and no further development in higher-risk MDS. Our royalty assumption is 12-19% for ex-US sales. Our projection for probability-weighted (50% for lower-risk MDS) sales of rigosertib reaches \$241M for US, and ex-US royalties are projected to reach \$55M by 2029. We use a discount rate of 10%, which we believe is appropriate given the probability-weighted sales projection.

Risks to Valuation

Risks to our valuation include the following:

- Binary clinical risk with Phase III readout of rigosertib in lower-risk MDS.
- Commercial and execution risks as a small company.
- Financing risk – ONTX ended 1Q14 with \$84.6M in cash, which together with anticipated milestone payments we estimate to be sufficient to fund operations into 2015, and the company may have additional financing needs before turning cash flow positive.



Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
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

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

In the past 12 months, the Firm has received compensation for providing investment banking services to Onconova Therapeutics, Inc. .

Leerink Partners LLC makes a market in Onconova Therapeutics, Inc.

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

©2014 Leerink Partners LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

Leerink Partners LLC Equity Research			
Director of Equity Research	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink
Associate Director of Research	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink
Healthcare Strategy	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink
Biotechnology	Howard Liang, Ph.D.	(617) 918-4857	howard.liang@leerink
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink
	Paul Matteis	(617) 918-4585	paul.matteis@leerink
	Richard Goss	(617) 918-4059	richard.goss@leerink
Life Science Tools and Diagnostics	Dan Leonard	(212) 277-6116	dan.leonard@leerink
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink
Pharmaceuticals/Major	Seamus Fernandez	(617) 918-4011	seamus.fernandez@leerink
	Ario Arabi	(617) 918-4568	ario.arabi@leerink
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink
Specialty Pharmaceuticals, Generics	Jason M. Gerberry, JD	(617) 918-4549	jason.gerberry@leerink
	Christopher W. Kuehnle, JD	(617) 918-4851	chris.kuehnle@leerink
Medical Devices, Cardiology & Orthopedics	Danielle Antalffy	(212) 277-6044	danielle.antalffy@leerink
	Richard Newitter	(212) 277-6088	richard.newitter@leerink
	Ravi Misra	(212) 277-6049	ravi.misra@leerink
Healthcare Services	Ana Gupte, Ph.D.	(212) 277-6040	ana.gupte@leerink
Healthcare Technology & Distribution	David Larsen, CFA	(617) 918-4502	david.larsen@leerink
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink
Supervisory Analysts	Robert Egan		bob.egan@leerink
	Amy N. Sonne		amy.sonne@leerink
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink

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