March 11, 2014

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK

ONCONOVA THERAPEUTICS, INC.

Phase III Oral Rigosertib Trial Planned for 2H:14 With a Biomarker Component

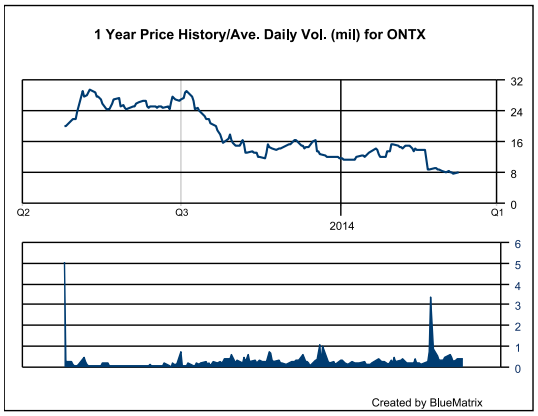
• **Bottom Line:** On its 4Q:13 call yesterday, ONTX provided updates on its 2014 catalysts surrounding oral and IV rigosertib. Based on discussions with the FDA, the company has designed a Phase III trial of oral rigosertib in lower-risk myelodysplastic syndrome (MDS), which it intends to initiate in 2H:14 after seeking approval for an SPA. According to management, the FDA has encouraged the company to consider the potential use of the methylation marker in the Phase III after a validation cohort. The company is also seeking a Type A meeting with the FDA, as well as meetings with EU regulatory agencies, to determine the next steps for advancing IV rigosertib in higher-risk MDS. However, given that the Phase III ONTIME trial in higher-risk patients failed to show a benefit in the primary endpoint of overall survival, we believe that it is appropriate for investor focus to shift to rigosertib's opportunity in the lower-risk population. We maintain our price target at $14.

• **Details of the Phase III trial in lower-risk MDS will be announced following SPA discussion with the FDA.** ONTX announced that it has designed a randomized, double-blind, placebo-controlled Phase III trial for oral rigosertib in lower-risk MDS patients who are transfusion-dependent and do not respond to erythropoietin-stimulating agents (ESAs). The company expects to finalize the details through the SPA process, which could take several months, and aims to initiate the trial in 2H:14. A validation cohort of 20 patients is also being enrolled in a Phase II trial to assess the prognostic methylation marker, and ONTX intends to include this biomarker in the Phase III trial, pending discussion with the FDA. While we await the final trial design, we continue to view the chances of Phase III success in lower-risk patients to be largely independent of ONTIME failure in higher-risk MDS.

• **ONTX plans to start Phase II dosing of oral rigosertib in combination with azacitadine in first-line MDS in 2H:14.** Following the completion of the dose-finding portion of the Phase I/II trial, the company expects to begin an enrollment of an expanded cohort in the selected dose. A total of 40 patients will be enrolled in the whole study, with additional updates provided in the second half of this year.

• **FDA approval of IV rigosertib in high-risk MDS based on subgroup analysis of ONTIME does not seem probable to us.** ONTX plans to hold discussions with the FDA to determine next steps following the failure of the ONTIME trial to show a survival benefit in the intent-to-treat (ITT) population. In what the company described as a hierarchical analysis, a significant OS improvement was seen in a subset of 184 patients who had not responded to prior hypomethylating agents (HMA) (either failed or progressed while on treatment). However, we believe that the FDA will likely be quite strict in considering this analysis. Full data from the ONTIME trial is expected at ASCO 2014.

Key Stats:	**(NASDAQ:ONTX)**
S&P 600 Health Care Index:	**1,311.16**
Price:	**$8.15**
Price Target:	$14.00
Methodology:	DCF analysis
52 Week High:	$31.13
52 Week Low:	$7.51
Shares Outstanding (mil):	21.4
Market Capitalization (mil):	$174.4
Book Value/Share:	$4.53
Cash Per Share:	$4.67
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	**$1.9**	**$1.0**	**$25.0**	**0.0**	**$27.9**	**($0.76)**	**($0.82)**	**$0.24**	**($0.88)**	**($2.18)**	**NM**
2014E - Old	--	--	--	--	$27.1	--	--	--	--	($2.36)	NM
2015E	--	--	--	--	$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.

4Q:13 Review and Model Update: Total 4Q:13 and FY13 revenue was $1.9M and $4.8M, above our estimates of $1.1M and $3.9M, respectively. Total 4Q operating expenses were ($16.5M), lower than our estimates of ($21.4M), driven by both lower R&D and SG&A spend. The company ended the year with $100M in cash, and 4Q EPS was ($0.68) versus our estimate of ($0.95). We have updated our model to account for these changes. Our price target remains at $14.

Onconova Expected Events

Event	Time	Comment
Rigosertib		
1st line, lower risk MDS (oral)	ASCO (May 30 - June 3, Chicago)	Additional Phase II data from ONTARGET trial expected at ASCO
2nd-line Higher-risk MDS (IV)	ASCO (May 30 - June 3, Chicago)	Full results of Phase III ONTIME trial expected at ASCO (late-breaker)
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)	H2:14	Initiation of Phase III trial design following FDA meeting regarding special protocol assessment (SPA)
1st line MDS in combo with azacitadine	H2:14	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 on path forward are ongoing
1st-line Lower-risk MDS (oral)	II	In discussions with FDA on Phase III design, seeking SPA; 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	Intitiated 3Q:13
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 valuation on ONTX is $14 based on DCF methodology. We assume rigosertib launches in lower-risk MDS in 2017 and no further development in higher-risk MDS. Our royalty assumption is 12-19% for ex-US sales. Our projection for peak penetration is 25% for lower-risk MDS. Our projection for probability-weighted (50% for lower-risk MDS) sales of rigosertib reaches $161M for US, and ex-US royalties reach $31M 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 has estimated pro forma cash of ~$100M, 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	Mar-13A	Jun-13A	Sep-13A	Dec-13A	2013A	Mar-14E	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements/Milestones										25,000		25,000	25,000	50,000	50,000	
Royalties													0	0	2,411	9,234
Sales													0	0	37,098	97,719
Total revenue	1,487	46,190	1,116	591	1,116	1,930	4,753	1,930	1,000	25,000	0	27,930	25,000	50,000	89,509	106,954
COGS														4,000	7,161	7,818
% of revenue														*8%*	*8%*	*8%*
R&D	22,624	52,762	12,756	10,047	15,293	12,086	50,182	12,328	12,574	12,826	13,082	50,810	54,946	56,594	58,292	60,040
SG&A	6,436	15,707	3,346	3,117	5,927	4,403	16,793	6,000	6,000	6,000	6,000	24,000	24,000	34,000	54,000	56,700
Total operating expenses	29,060	68,469	16,102	13,164	21,220	16,489	66,975	18,328	18,574	18,826	19,082	74,810	78,946	94,594	119,452	124,558
Net income (loss) from operations	(27,573)	(22,279)	(14,986)	(12,573)	(20,104)	(14,559)	(62,222)	(16,398)	(17,574)	6,174	(19,082)	(46,880)	(53,946)	(44,594)	(29,943)	(17,605)
Change in fair value of warrant liability	1,287	367	14	(2)	(31)	61	42									
Interest expense	(19)	(8,608)	0	(2)	(1)	(1)	(4)									
Other income, net	11	608	127	15	47	(126)	63	0	0	0	0	0	0	0	0	0
Net income (loss) before income taxes	(26,294)	(29,912)	(14,845)	(12,562)	(20,089)	(14,625)	(62,121)	(16,398)	(17,574)	6,174	(19,082)	(46,880)	(53,946)	(44,594)	(29,943)	(17,605)
Provision (benefit) for income taxes	0	0	0	0	432	3	435	0	0	0	0	0	0			
Tax rate																
Net loss attributable to non-controlling interest						13										
Net income (loss)	(26,294)	(29,912)	(14,845)	(12,562)	(20,521)	(14,615)	(62,556)	(16,398)	(17,574)	6,174	(19,082)	(46,880)	(53,946)	(44,594)	(29,943)	(17,605)
Accretion of preferred stock	(4,020)	(3,953)	(1,019)	(1,032)	(269)	0	(2,320)	0	0	0	0	0	0			
Net income (loss) to common stockholders	(30,314)	(33,865)	(15,864)	(13,594)	(20,790)	(14,615)	(64,876)	(16,398)	(17,574)	6,174	(19,082)	(46,880)	(53,946)	(44,594)	(29,943)	(17,605)
Net loss per share	**(14.18)**	**(2.67)**	**(1.03)**	**(0.64)**	**(0.97)**	**(0.68)**	**(6.12)**	**(0.76)**	**(0.82)**	**0.24**	**(0.88)**	**(2.18)**	**(1.69)**	**(1.37)**	**(0.78)**	**(0.45)**
Basic shares	2,137	12,669	15,446	21,389	21,404	21,419	10,594	21,462	21,505	21,548	21,591	21,527	31,957	32,596	38,248	39,013
Dilutive shares			15,446	24,186	24,428	24,672		25,166	25,669	26,182	26,706	25,931	36,190	38,000	44,899	47,144

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.

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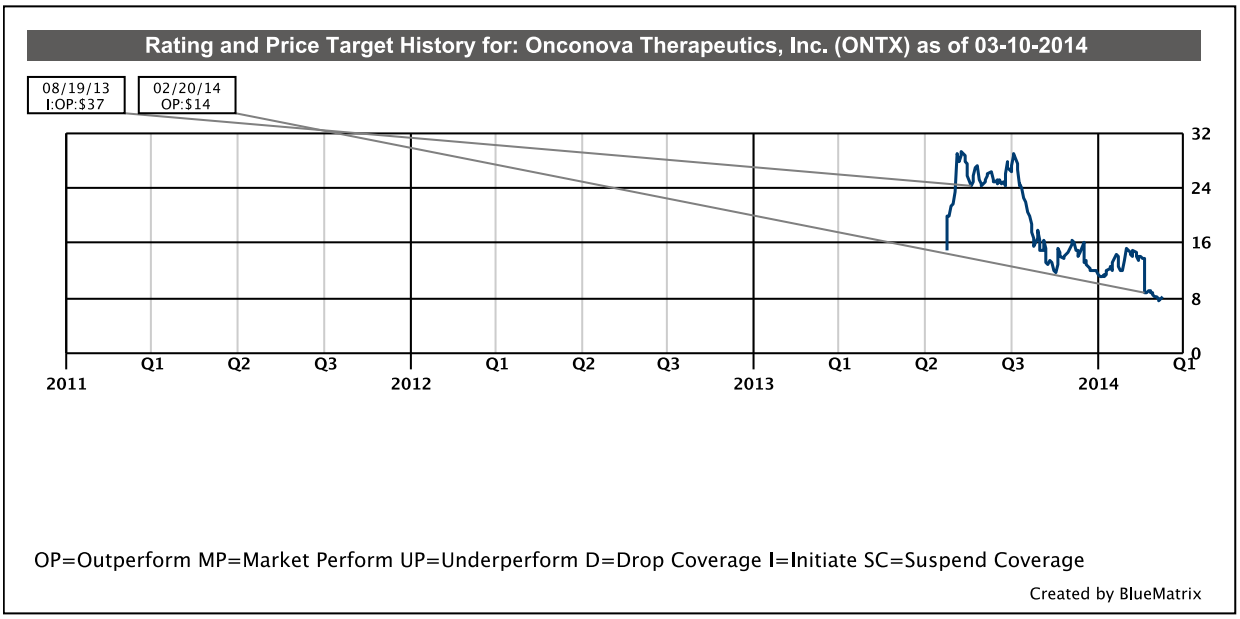

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	118	64.50	30	25.00
HOLD [MP]	65	35.50	2	3.00
SELL [UP]	0	0.00	0	0.00

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

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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.

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