November 5, 2013

OUTPERFORM

Reason for report:

PROPRIETARY INSIGHTS

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LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

ONCONOVA THERAPEUTICS, INC.

Analysis Provides Comfort about Contribution of ESA to Rigosertib Response

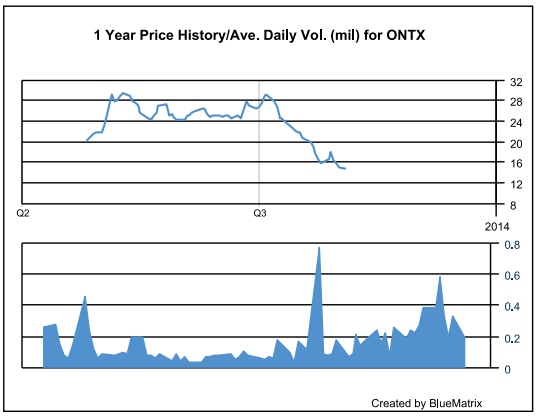
• **Bottom Line:** There have been questions raised about Phase II data of rigosertib in the lower-risk MDS (interim results presented at ASCO this year) regarding the contribution of erythropoietin-stimulating agent (ESA) which was used in most responders. Our analysis suggests that ESA is unlikely to be primarily responsible for the transfusion independence responses seen due to 1) limited use of ESA relative to the duration of response, 2) general lack of correlation between ESA use and transfusion independence, and 3) a patient population that appeared unlikely to respond to ESA alone. We continue to believe that there has been a strong signal of rigosertib in lower-risk MDS setting which could provide enhancement of upside as well as down-side protection heading into Phase III data in higher-risk MDS (4Q:13/1Q:14).

• **More than half of the responders received only one dose or no ESA and the pattern of response does not appear to correlate with ESA administration, suggesting that the transfusion independence response was not due to ESA.** According to a MEDACorp hematologist, MDS patients whose hemoglobin level falls below certain level (e.g., 9 g/dl) typically are given ESA and almost all of the responders (11/13) received an ESA at some point in the trial. However, based on our analysis the duration and timing of this administration did not correlate closely with the achievement of transfusion independence in most patients (see table and chart that follows).

• **Baseline characteristics of the Phase II patients suggest they would be unlikely to respond to ESAs alone.** All 26 patients who received intermittent rigosertib had been transfused with four units of red blood cells (RBC) within the two weeks prior to enrollment and these patients appeared to have a low probability of responding to ESA alone based on published studies. In addition, eleven of the 13 responders received prior ESAs yet remained transfusion dependent.

• **ASH data could provide better clarity.** We expect updated results from the lower-risk MDS trial at ASH (abstract to be released on 11/7) with data on most of the 60 enrolled patients (vs. 34 evaluable at ASCO). We expect data on a cohort of patients who do not receive ESAs for the first three months of the study to help address the question of ESA contribution to response. In addition, we expect genomic signature data that could help select patients that are likely to respond to rigosertib.

Key Stats:	**(NASDAQ:ONTX)**
S&P 600 Health Care Index:	**1,207.27**
Price:	**$14.86**
Price Target:	$37.00
Methodology:	DCF with 10% discount rate
52 Week High:	$31.13
52 Week Low:	$14.53
Shares Outstanding (mil):	2.6
Market Capitalization (mil):	$38.7
Book Value/Share:	$37.17
Cash Per Share:	$31.28
Dividend (ann):	$0.00
Dividend Yield:	0.0%



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2012A	--	--	--	--	$46.2	--	--	--	--	($2.67)	NM
2013E	$1.1A	$1.1	$1.1	$1.1	$4.5	($1.03)A	($0.79)	($0.71)	($0.72)	($3.18)	NM
2014E	--	--	--	--	$2.1	--	--	--	--	($3.34)	NM

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

Please refer to pages 9 - 11 for Important Disclosures, Price Charts and Analyst Certification.

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INVESTMENT THESIS

ONTX is a late-stage story with robust clinical news flow in the next 6-9 months including pivotal Phase III (ONTIME) data of IV rigosertib in higher-risk second-line myelodysplastic syndrome (MDS). Although the Phase III ONTIME readout is clearly a binary event and there is considerable risk, we believe risk/reward remains favorable. In Phase II trials of higher-risk MDS patients, rigosertib demonstrated good bone marrow (BM) response but more modest hematological improvements. Although we are not aware of data clearly showing a correlation of BM response and survival, we believe the clear correlation of BM blast percentage and survival in historical data is supportive. Another positive consideration is that rigosertib is being compared to best supportive care, and based on our analysis, we do not believe low-dose ara-C will have a meaningful contribution. Although the study is not large for a survival study (270 patients targeted, nearly 300 enrolled), the number of events used for analysis (at least 223) looks reasonable in comparison to the successful AZA-001 study in the first-line setting (195 events). MEDACorp key opinion leaders generally peg the probability of success to be at least 50%, and as high as 80%. We believe 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 response such as transfusion independence was seen. MEDACorp KOLs view rigosertib data in lower-risk patients to be even stronger than in higher-risk MDS. We believe upcoming ASH data could further solidify the profile. We believe there are limited expectations for pancreatic cancer interim Phase II/III readout in 4Q:13/1Q:14; therefore risk/reward is favorable. We find signals of single-agent activity in head and neck cancer intriguing. ONTX is one of the minority of biotech companies that have been able to maintain full US rights to their lead compound near the finish line. If the Phase III is positive, we believe rigosertib will be an attractive asset to potential acquirers due to retained economics as well as a pipeline of additional indications.

Duration and timing of rigosertib response do not appear to correlate closely with ESA administration, suggesting that the transfusion independence response was not due to ESA. At the time of the ASCO poster presentation in June, 43 patients had been enrolled in the trial (26 receiving intermittent rigosertib (days 1-14 of a 21 days cycle) and 8 receiving the discontinued continuous dosing regimen. Of the 26 intermittently-dosed patients who were evaluable for transfusion independence (defined as no RBC transfusion for at least eight consecutive weeks), 13 were determined to be responders. According to a MEDACorp hematologist, MDS patients whose hemoglobin level falls below certain level (e.g., 9) typically are given ESA and almost all of the responders (11/13) received an ESA at some point in the trial. However, based on our analysis the duration and timing of this administration did not correlate closely with the achievement of transfusion independence in most patients (see table and chart below).

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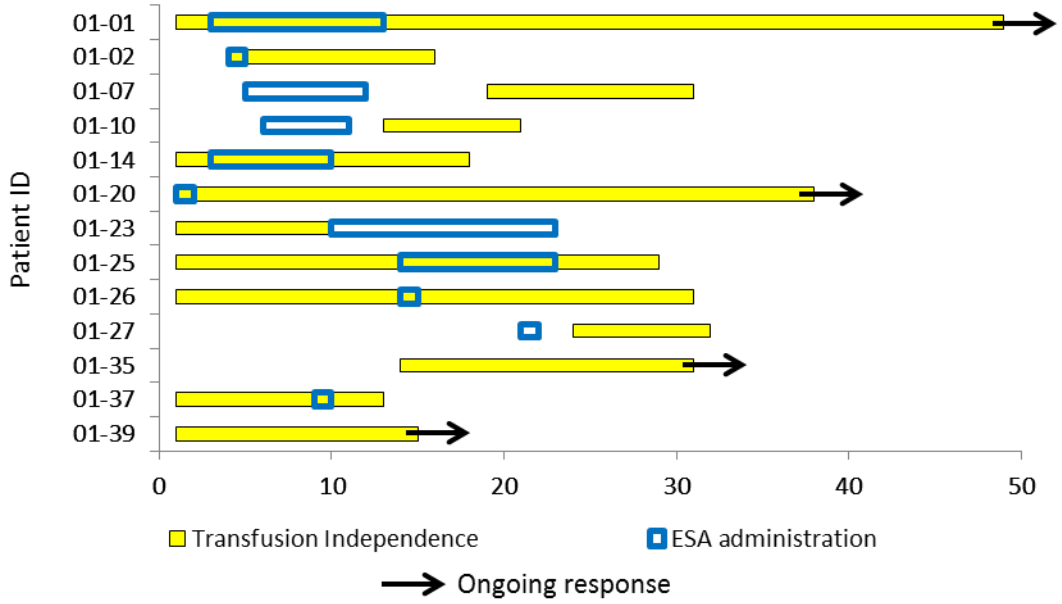
Duration of transfusion independence and ESA administration



Transfusion Independence Responders – Outcomes (weeks)

PID	Response week		ESA Treatment week		
	Onset	*Duration*	*Start*	*Stop*	
01-01	**1**	**48+**	**3**	**12**	Early response
01-02*	4	12	4	4	
01-07*	19	12	5	11	Delayed response
01-10*	13	8	6	10	**> 6months of TR**
01-14	1	17	3	9	*Potential synergy with ESA
01-20	**1**	**37+**	**1**	**1**	
01-23	1	9	10	22	
01-25	**1**	**28**	**14**	**22**	
01-26	**1**	**30**	**14**	**14**	
01-27*	24	8	21	21	
01-35	14	17+	No	No	
01-37	1	12	9	9	
01-39	1	14+	No	No	

Source: Raza et al (ASCO 2013 poster), Leerink Swann analysis

Rigosertib may have synergy with ESAs. The investigators of the trial identified four patients (01-02, 01-07, 01-10, and 10-27) and suggested that rigosertib had "potential synergy with ESA" due to the fact that their periods of transfusion independence started with or shortly after its

administration. Additionally, it is possible that other patients may have had their duration of transfusion independence extended by addition of an ESA.

Baseline characteristics of the Phase II patients suggest they would be unlikely to respond to ESAs alone. All 26 patients who received intermittent rigosertib had been transfused with four units of RBC within the two weeks prior to enrollment (as required by the inclusion criteria). Eleven of the 13 responders received prior ESAs, although the corresponding number was not reported for the 13 rigosertib non-responders (22 of the 43 total patients in the trial received prior ESAs).

According to a predictive model developed by Hellstrom-Lindberg et al. (1997, British Journal of Haematology, 99, 344–351), patients who receive two or more units of RBC per month would be expected to have poor (7%) or intermediate (23%) responses to an ESA in combination with a granulocyte-colony stimulating factor (G-CSF). In a 2003 study, Hellstorm-Lindberg et al assessed MDS patients who fell in the good and intermediate categories and found that only 15% of those who required two or more units per month responded to ESA+G-CSF therapy. Thus, even patients in the rigosertib trial who did not receive prior ESAs would be unlikely to respond to ESAs alone.

Baseline characteristics of rigosertib-responders in the Phase II trial

Transfusion Independence Responders - Pre-Treatment Characteristics

PID	IPSS	Cytogenetics	Prior Trt	Prior ESA	EPO mU/mL	Prior RBC*
01-01	0.5	-Y	Len	Yes	51	4
01-02	0.5	18 abn	Len	Yes	35	4
01-07	0.5	Normal	No	Yes	117	4
01-10	0	Normal	No	Yes	32	4
01-14	0.5	Normal	Len	Yes	51	4
01-20	0	Normal	Len	No	128	4
01-23	1	-Y	No	Yes	14	4
01-25	1	Complex	No	No	15	4
01-26	0.5	del20q	Aza	Yes	361	4
01-27	0	Normal	Len	Yes	31	4
01-35	1	+8	Aza	Yes	47	4
01-37	0	Normal	No	Yes	236	4
01-39	0.5	del13q	Aza/Len	Yes	216	4

*Prior RBC = Number of RBC units administered within 8 weeks before baseline

Source: Raza et al (ASCO 2013 poster), Leerink Swann analysis

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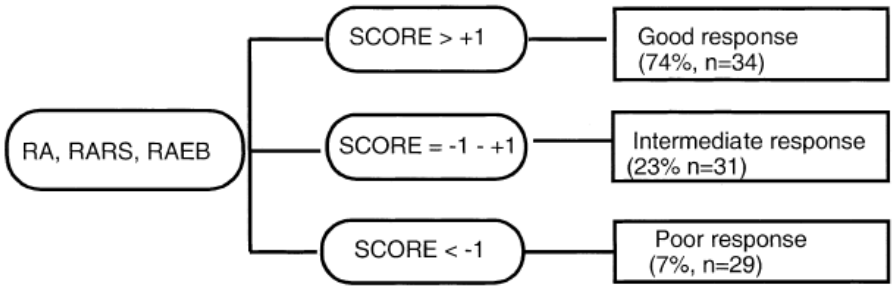
Predictive model of response to ESA+G-CSF therapy



Treatment response criteria

CR Stable Hemoglobin >11.5 g/dl

PR Increase in Hb with >1.5 g/dl or total stop in RBC transf.

Treatment response score

S-EPO U/l	<100	+ 2
	100-500	+ 1
	>500	- 3
Transf. U RBC / m	<2 units / m	+ 2
	= or >2 units / m	- 2

Table II. Category variables in responding and non-responding patients (univariate analysis).

Variable	Subgroups*	Response rates (CR + PR)	*P*-value
Sex (F/M)†	24 female/29 male	46%/38%	0·76
Diagnosis	15 RA/21 RARS/17 RAEB	47%/52%/24%‡	0·18
Diagnosis	36 RARS + RA/17 RAEB	50%/24%‡	0·07
Karyotype risk§	30 low/12 int/8 high	47%/58%/13%	0·12
IPSS¶	16 low/26 int-1/5 int-2/3 high	56%/46%/0%/33%	0·17
Transfusions (yes/no)	36 no/17 yes	71%/28%	0·008
Transfusion need**	33 no need or < 2/20 ≥ 2	58%/15%	0·006
Transfusion need	17 0/16 < 2/20 ≥ 2	71%/44%/15%	0·003
Serum Epo‡	30 < 100 U/23 ≥ 100 U/l	56%/22%	0·02
Serum Epo	40 < 200 U/11 ≥ 200 U/l	45%/18%	0·16
Serum Epo‡	12 100–200/8 200–500	25%/25%	1·0
Predictive group††	31 good/22 intermediate	61%/14%	0·001

*No. of patients per subgroup.
†Male/female.
‡U/l. Three patients had S-Epo > 500 U/l.
§Karyotype risk group according to IPSS; low, intermediate, high.
¶IPSS, International Prognostic Scoring System (Greenberg *et al.*, 1997). Low, low risk; int-1, intermediate 1 risk; int-2, intermediate 2 risk; high, high risk. Note, only five patients in Int-2 and -3 in high-risk group, see Table I.
**Units of RBC/month.
††Predictive group according to Hellström-Lindberg *et al* (1997b).

Note: Patients in Table II belong only to the Good and Intermediate predictive groups
Source: Hellstorm-Lindberg et al (2003, British Journal of Haematology, 2003, 120, 1037–1046.)

ONTX Expected Events

Event	Time	Comment
Rigosertib		
2nd line, higher risk MDS Phase III topline results	4Q:13 - 1Q:14	270+ patients completed enrollment during Dec 2010 - May 2013. Data more likely in 1Q:14.
1st line, lower risk MDS Phase II update	ASH (Dec 7-10, New Orleans)	Data on ~60 patients expected; data on 34 patients presented at ASCO 2013
1st line pancreatic cancer Phase II/III interim futility / survival analysis	4Q:13 - 1Q:14	Data more likely in 4Q:13

Source: Company reports and Leerink Swann LLC

ONTX Product Pipeline

Agent	Phase	Status/Anticipated Milestones
Rigosertib Single-agent		
2nd-line Higher-risk MDS (IV)	III	Top-line survival results 4Q:13-1Q:14
1st-line Lower-risk MDS (oral)	II	Initital response data 2Q:13
Head and Neck Cancer (oral)	II	Complete Phase II enrollment 2H:14
Rigosertib in Combination		
1st-line Pancreatic Metastatic (IV) Gemcitabine Comination	III	Interim survival analysis 4Q:13-1Q:14
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 and Leerink Swann LLC

VALUATION

Our 12-month valuation on ONTX shares is $37 based on DCF methodology. We assume rigosertib launches in higher-risk MDS in 2015 and in lower-risk MDS in 2017. Our royalty assumption is 12-19% for ex-US sales. Our projection for peak penetration is 30% for high-risk MDS and 25% for low-risk MDS. Our projection for probability-weighted (60% for higher-risk and 50% for lower-risk MDS) sales of rigosertib reaches $394M for US, and ex-US royalties reach

$75M 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 higher-risk MDS. Although Phase II demonstrated bone marrow response, full partial or complete responses by traditional definition were limited, and it is not clear that bone marrow response would predict survival benefit. In addition, although the survival observed in the Phase II compared favorably to historical control, such comparisons are difficult and have significant caveats.

Commercial and execution risks as a small company. The current continuous infusion dosing regimen for the IV formulation may present a challenge.

Financing risk – ONTX has estimated pro forma cash of ~$110M, which we estimate to be sufficient to fund operations through the end of 2014, and the company may have additional financing needs before turning cash flow positive.

ONTX Income Statement ($000)	2011A	2012A	Mar-13A	Jun-13E	Sep-13E	Dec-13E	2013E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements												
Royalties									2,681	6,914	21,868	32,739
Sales									44,678	79,013	168,213	242,512
Total revenue	1,487	46,190	1,116	1,116	1,116	1,116	4,464	2,116	47,359	85,927	190,080	275,251
COGS										6,874	15,206	19,401
% of revenue										*8%*	*8%*	*8%*
R&D	22,624	52,762	12,756	12,884	13,012	13,143	51,794	59,478	61,263	63,100	64,993	66,943
SG&A	6,436	15,707	3,346	3,379	3,413	3,447	13,586	30,000	67,017	79,013	75,696	84,879
% of revenue									*150%*	*100%*	*45%*	*35%*
Total operating expenses	29,060	68,469	16,102	16,263	16,426	16,590	65,381	75,080	128,279	148,988	155,895	171,223
Net income (loss) from operations	(27,573)	(22,279)	(14,986)	(15,147)	(15,310)	(15,474)	(60,917)	(72,964)	(80,921)	(63,061)	34,185	104,028
Change in fair value of warrant liability	1,287	367	14	0	0	0	14					
Interest expense	(19)	(8,608)	0	0	0	0	0					
Other income, net	11	608	127	0	0	0	127	0	0	0	0	0
Net income (loss) before income taxes	(26,294)	(29,912)	(14,845)	(15,147)	(15,310)	(15,474)	(60,776)	(72,964)	(80,921)	(63,061)	34,185	104,028
Provision (benefit) for income taxes	0	0	0	0	0	0	0	0	0			
Tax rate												
Net income (loss)	(26,294)	(29,912)	(14,845)	(15,147)	(15,310)	(15,474)	(60,776)	(72,964)	(80,921)	(63,061)	34,185	104,028
Accretion of preferred stock	(4,020)	(3,953)	(1,019)	(1,797)	0	0	(2,816)	0	0			
Net income (loss) to common stockholders	(30,314)	(33,865)	(15,864)	(16,944)	(15,310)	(15,474)	(63,592)	(72,964)	(80,921)	(63,061)	34,185	104,028
Net loss per share	**(14.18)**	**(2.67)**	**(1.03)**	**(0.79)**	**(0.71)**	**(0.72)**	**(3.18)**	**(3.34)**	**(3.52)**	**(2.61)**	**1.23**	**3.57**
Basic shares	2,137	12,669	15,446	21,389	21,496	21,604	19,984	21,875	22,969	24,117	25,323	26,589
Dilutive shares			15,446	24,186	24,428	24,672	22,183	24,919	25,168	26,426	27,748	29,135

Source: Company Reports and Leerink Swann

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

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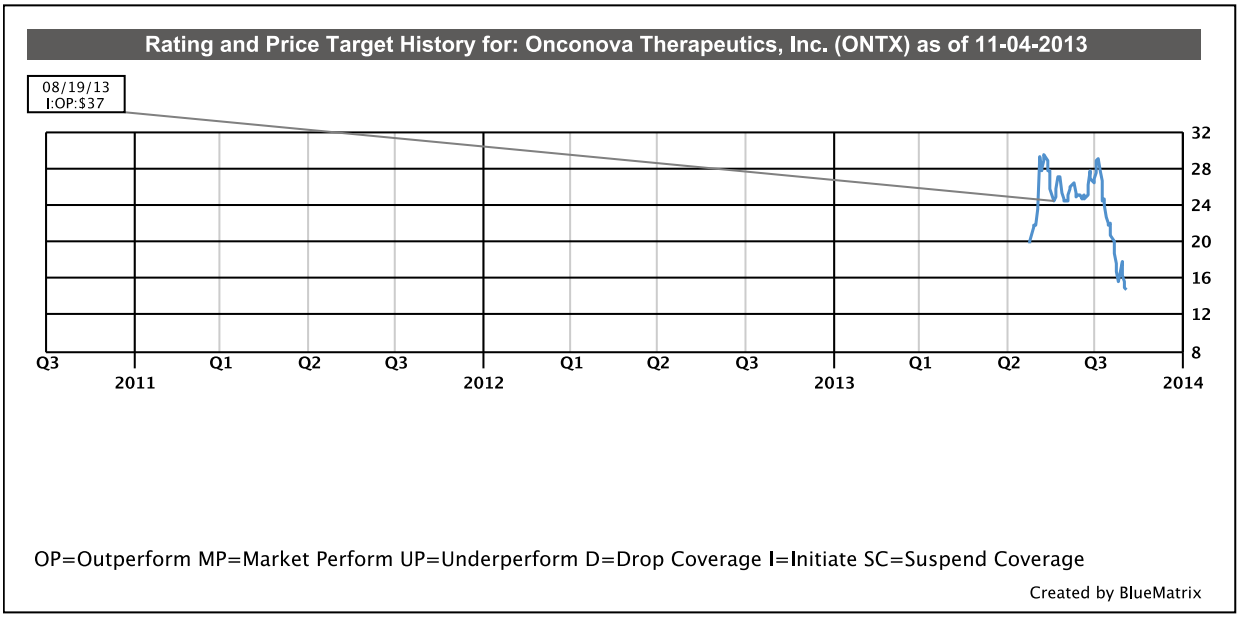

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	111	64.90	27	24.00
HOLD [MP]	60	35.10	0	0.00
SELL [UP]	0	0.00	0	0.00

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 Swann LLC makes a market in Onconova Therapeutics, Inc.

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

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