March 21, 2014

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK

AMBIT BIOSCIENCES CORP.

4Q:13 Call Highlights Quizartinib Clinical Development Strategy

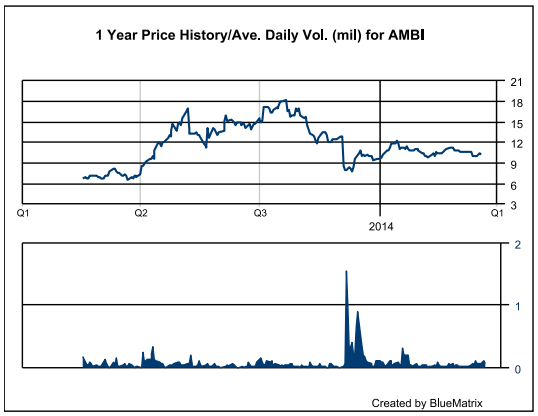
• **Bottom Line:** On the 4Q:13 earnings call, AMBI provided more color on the upcoming QUANTUM-R Phase III registration trial to evaluate quizartinib in FLT3+ acute myeloid leukemia (AML) patients. While the company initiates QUANTUM-R (in 2Q:14) which is expected to progress toward interim and final data in 2H:15 and 1Q:16 respectively, potential Phase III data this year from a less potent FLT3 inhibitor midostaurin (NVS [OP]) may be of interest. While long-term focus remains QUANTUM-R overall survival (OS) outcome, near-term catalysts include Phase I data in maintenance treatment after stem cell transplant, which could provide color on duration of remission while on quizartinib maintenance that in turn could have a read-through to the Phase III. AMBI is also seeking an ex-US partnership and has several trials to expand quizartinib market potentials into earlier lines as well as patient groups with different age or fit conditions. Our price target for AMBI remains $14.

• **More color on quizartinib Phase III trial.** AMBI will initiate quizartinib Phase III trial in FLT3-ITD+ AML patients in 2Q:14 (a slight delay vs. our expectation of 1Q:14). Majority of the Phase III sites are the same as the Phase II trials. Based on the Phase II trials where 35-40% patients underwent stem cell transplant, management estimated the same or slightly higher rate of patients could undergo stem cell transplant in the Phase III trial since patients will be in 1st salvage (vs. 2nd salvage for the Phase II trials), while estimated 15-20% patients will undergo stem cell transplant for the chemo control arm. Maintenance therapy with quizartinib should be triggered within 30-100 days post-transplant. For the adaptive design with event-driven interim analysis (140 events, Hazard Ratio of 0.65-0.87 and 0.70 triggers adaptive design, 90% powered), interim re-sizing is driven by predetermined criteria, which are not disclosed to AMBI, but shared by the Data Safety Monitoring Board (DSMB) with independent statisticians..

• **Seeking Ex-US partnership but likely maintain the US rights.** AMBI estimated ~$40M costs for the quizartinib Phase III trial. The aim of the partnership would include upfront and milestone payment as well as co-funding of ongoing and additional Phase III trials, potentially extending the cash runway.

• **Upcoming catalysts.** Near-term catalysts include quizartinib Phase I maintenance data in AML, which are expected at an upcoming medical conferences (likely ASCO, EHA or ASH). Following the planned quizartinib Phase III initiation in 2Q:14 (a press release with the first enrollment), interim analysis is expected in 2H:15 with top-line data anticipated in 1Q:16 (slight delay vs. prior guidance of YE:15). Additionally, greater visibility is expected in 2014 for early candidates AC410 (JAK2 inhibitor, entering Phase II) and AC708 (CSF1R inhibitor, entering a clinical trial).

Key Stats:	**(NASDAQ:AMBI)**
S&P 600 Health Care Index:	**1,312.16**
Price:	**$10.21**
Price Target:	$14.00
Methodology:	Prob. weighted NPV, 10% discount rate
52 Week High:	$21.44
52 Week Low:	$6.22
Shares Outstanding (mil):	17.9
Market Capitalization (mil):	$182.8
Book Value/Share:	$(0.46)
Cash Per Share:	$3.97
Dividend (ann):	$0.00
Dividend Yield:	0.0%



Dec YE	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	**$6.6**	**$11.5**	**$7.7**	**$1.3**	**$27.1**	**($3,019.30)**	**$0.45**	**($0.34)**	**($0.11)**	**($1.19)**	**NM**
2014E - New	**$3.5**	**$3.5**	**$3.5**	**$3.5**	**$14.0**	**($0.41)**	**($0.41)**	**($0.41)**	**($0.40)**	**($1.62)**	**NM**
2014E - Old	--	--	--	--	$15.0	--	--	--	--	($1.58)	NM
2015E	--	--	--	--	$12.0	--	--	--	--	($1.45)	NM

Source: Company Information and Leerink Partners LLC Research
Revenues in millions.
GAAP EPS. May 2013 IPO.

INVESTMENT THESIS

Based on MEDACorp key opinion leader (KOL) feedback, we believe AMBI's lead candidate quizartinib is the best FLT3 (FMS-like tyrosine kinase-3) inhibitor in development and best currently available targeted agent for acute myeloid leukemia (AML), a devastating hematological cancer with few options and high unmet need. Quizartinib is a wholly owned, best-in-class FLT3 inhibitor, late-stage asset in an area with limited competition. It has been tested in over 400 patients and has shown a promising efficacy and safety profile. MEDACorp KOLs do not view QTc prolongation associated with quizartinib to be limiting. The end of Phase II meeting with the FDA suggested that CRi as a surrogate endpoint is not suitable for an accelerate approval. Although near-term upside was removed for AMBI, we believe that long-term potential remains since Phase III would be required for a full approval.

Model update. AMBI reported $1.3M in revenue and ($2M) in net loss, vs. our estimate of $2M and ($7M), respectively. The company ended the quarter with $71M cash, sufficient to support operation through quizartinib Phase III trial (1Q:16), in our view. We update our model to reflect these changes. As a result, our 2014 revenue projection changes from $15M to $14M and EPS estimate from ($1.58) to ($1.62).

AMBI Upcoming Catalysts

Compound	Timing	Event
Quizartinib (FLT3i)	2Q:14	Phase III initiation in R/R AML with Flt3-ITD
	2H:15	Enrollment completion, Phase III interim analysis
	1Q:16	Phase III top line data
	Medical conference in 2014	Phase I post-transplant maintenance trial data
AC708 (CSF1Ri)	2Q:14	IND submission

Source: Company Reports and Leerink Partners

AMBI Pipeline

Indication	Status	Comments
Quizartinib (Flt3 inhibitor)		
R/R AML	Phase II	CRc rate was 46% with 35% bridged to a HSCT. Full data from 30mg and 60mg to be presented at ASH 2013.
R/R AML with Flt3-ITD	Phase III	Phase III in R/R AML with Flt3-ITD to be initiated in early '14.
Front line AML	Phase I	Frontline in combination with chemotherapy to be presented at ASH 2013. In all AML pts.
Post-HSCT maintenance	Phase I	In all AML patients
AC410/AC430 (JAK2 inhibitor)		
Inflammation	Phase I	Completed Phase I
AC708 (CSF1R inhibitor)		
Oncology/Inflammation	Preclinical	IND submission in 2Q:14
CEF-32496 (BRAF inhibitor)		
Oncology	Preclinical	

Source: Company Reports and Leerink Partners

VALUATION

Our $14 valuation for AMBI is derived from probability-weighted NPV valuation methodology. Our projection for peak penetration is 50% in the U.S. and Japan and 45% in the EU. Our projection for probability-weighted (60%) sales reaches $450M by 2029, one year after patent expiration. We use a discount rate of 10%, which we believe is appropriate given probability-weighted sales projection.

RISKS TO VALUATION

- Clinical risk – although Phase II data are promising, these are single-arm studies and the controlled randomized Phase III trial may fail to show OS benefit vs. chemo therapy.
- Commercial risk – quizartinib may face competition from other drugs targeting Flt3-ITD.
- Financing risk – AMBI may not have sufficient cash to support operations through profitability.

AMBI Income Statement	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	17,633	6,592	11,547	7,678	1,276	27,093	3,500	3,500	3,500	3,500	14,000	12,000			
Quizartinib sales													2,020	17,752	41,045
Total revenue	17,633	6,592	11,547	7,678	1,276	27,093	3,500	3,500	3,500	3,500	14,000	12,000	2,020	17,752	41,045
COGS													162	1,420	3,284
% of revenue													*8%*	*8%*	*8%*
R&D	36,731	9,005	6,664	4,484	6,131	26,284	8,000	8,000	8,000	8,000	32,000	32,960	33,949	34,967	36,016
SG&A	6,550	1,776	2,197	3,076	3,293	10,342	3,000	3,000	3,000	3,000	12,000	14,400	25,000	30,179	32,836
% of revenue														*170%*	*80%*
gain on sale of kinase profiling services	(2,497)	0	0	(2,500)	0	(2,500)	0	0	0	0	0	0	0	0	
Total operating expenses	40,784	10,781	8,861	5,060	9,424	34,126	11,000	11,000	11,000	11,000	44,000	47,360	59,110	66,566	72,136
Net income (loss) from operations	(23,151)	(4,189)	2,686	2,618	(8,148)	(7,033)	(7,500)	(7,500)	(7,500)	(7,500)	(30,000)	(35,360)	(57,090)	(48,814)	(31,091)
Interest expenses	(1,737)	(162)	(108)	(53)	0	(323)	0	0	0	0	0	0			
Other income	29	7	5	1	0	13	0	0	0	0	0	0			
Change in fair value of derivative liabilies	(2,291)	(3,957)	2,577	(8,665)	6,103	(3,942)	0	0	0	0	0	0			
Total other income (expenses)	(3,999)	(4,112)	2,474	(8,717)	6,103	(4,252)	0	0	0	0	0	0	0	0	0
Net income (loss) before income taxes	(27,150)	(8,301)	5,160	(6,099)	(2,045)	(11,285)	(7,500)	(7,500)	(7,500)	(7,500)	(30,000)	(35,360)	(57,090)	(48,814)	(31,091)
Provision (benefit) for income taxes	(121)	1	0	(30)	0	(29)	0	0	0	0	0	0			
Tax rate															
Net income (loss)	(27,029)	(8,302)	5,160	(6,069)	(2,045)	(11,256)	(7,500)	(7,500)	(7,500)	(7,500)	(30,000)	(35,360)	(57,090)	(48,814)	(31,091)
Non-controlling interest	382	73	(12)	0	0	61	0	0	0	0	200	200			
Net income (loss) attributable to AMBI	(26,647)	(8,229)	5,148	(6,069)	(2,045)	(11,195)	(7,500)	(7,500)	(7,500)	(7,500)	(29,800)	(35,160)	(57,090)	(48,814)	(31,091)
Other comprehensive income															
Accretion to redemption value of reddemable convertible preferred stock	(3,161)	(2,319)	(1,315)	0	0	(3,634)	0	0	0	0	0	0			
Change in fair value of redeemable non-controlling interest	(854)	(1,499)	3,246	0	0	1,747	0	0	0	0	0	0			
Net income to preferred stockholders			(3,457)				0	0	0	0	0				
Net loss to common stockholders	**(30,662)**	**(12,047)**	**3,622**	**(6,069)**	**(2,045)**	**(13,082)**	**(7,500)**	**(7,500)**	**(7,500)**	**(7,500)**	**(29,800)**	**(35,160)**	**(57,090)**	**(48,814)**	**(31,091)**
Foreign currency translation		(133)	3	0	(243)	(373)	0	0	0	0	0				
Net loss per share	**(16,591.99)**	**(3,019.30)**	**0.45**	**(0.34)**	**(0.11)**	**(1.19)**	**(0.41)**	**(0.41)**	**(0.41)**	**(0.40)**	**(1.62)**	**(1.45)**	**(1.87)**	**(1.52)**	**(0.85)**
Basic shares	2	4	8,055	17,877	17,919	11,024	18,098	18,279	18,462	18,647	18,372	24,290	30,505	32,030	36,631
Dilutive shares			9,752	21,208	21,478	17,479	21,908	22,346	22,793	23,249	22,574	28,702	35,137	36,894	41,739

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

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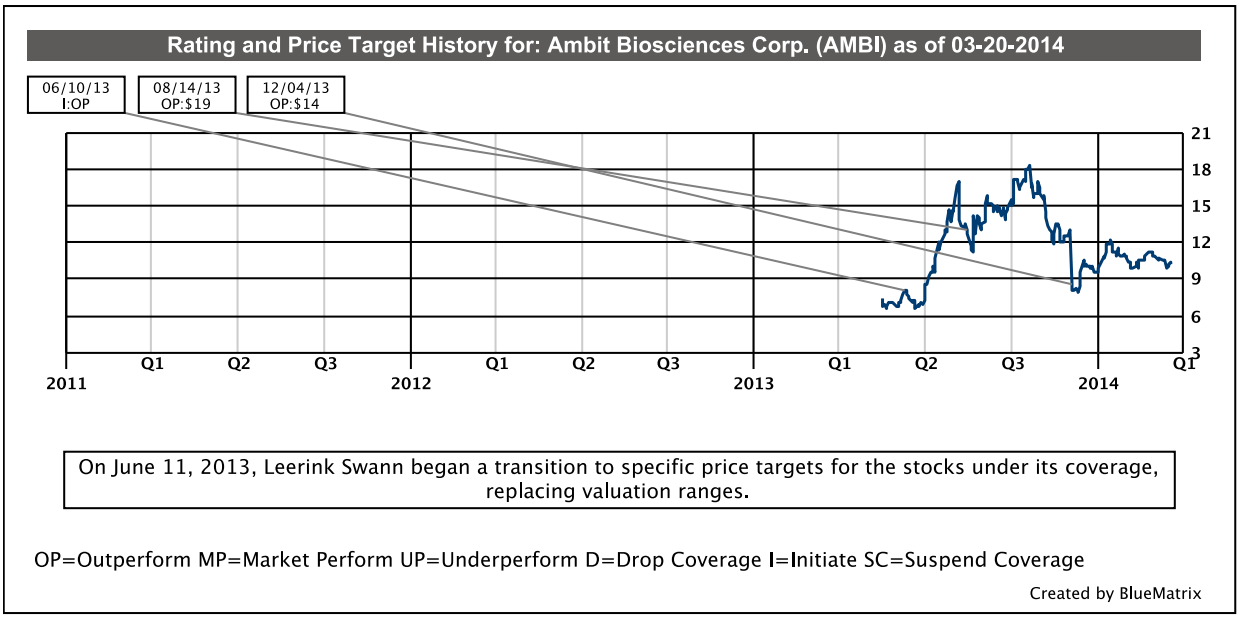
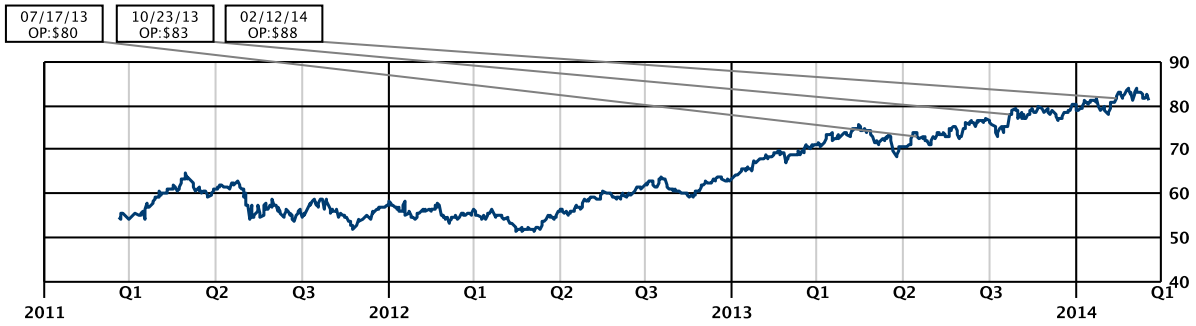


Rating and Price Target History for: Novartis AG (NVS) as of 03-20-2014



Leerink Swann initiated coverage of NVS with an Outperform rating on November 9, 2010. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

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 Consulting LLC, an affiliate of Leerink Partners, is a provider of evidence-based strategy and consulting to the healthcare industry.

In the past 12 months, the Firm has received compensation for providing investment banking services to Ambit Biosciences Corp. .

Leerink Partners LLC makes a market in Ambit Biosciences Corp.

Leerink Partners LLC is willing to sell to, or buy from, clients the common stock of Novartis AG on a principal basis.

In the past 12 months, an affiliate of the Firm, Leerink Swann Consulting LLC, has received compensation for providing non-securities services to: Novartis AG.

Leerink Partners LLC has acted as the manager for a public offering of Ambit Biosciences Corp. in the past 12 months.

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