May 7, 2014

OUTPERFORM

Reason for report:

EARNINGS

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AMBIT BIOSCIENCES CORP.

1Q:14 Call -- Phase III On Track; Post-transplant Maintenance Could Help

• **Bottom Line:** On its 1Q:14 earnings call, AMBI highlighted recent initiation of the Phase III QUANTUM-R trial in relapsed/refractory FLT3-ITD positive AML patients and clinical presentations at two upcoming medical conferences (ASCO [5/30-6/3] and EHA [6/12-15]). While long-term focus remains quizartinib QUANTUM-R overall survival outcome (interim data in 2H:15, topline data in 1Q:16), near-term catalysts could include data presentation at ASCO from Phase IIb full data and bridge-to-transplant studies, data on post-transplant maintenance which appeared to be quite convincing, potential Phase III data from NVS (OP)'s midostaurin, a less potent FLT3 inhibitor, and potential licensing of quizartinib for OUS.

• **QUANTUM-R trial initiation on track, interim data anticipated in 2H:15.** The Phase III QUANTUM-R trial will evaluate quizartinib monotherapy vs. salvage chemotherapy in R/R FLT3-ITD positive AML patients. Initial dose includes either 20mg or 30mg. As stated previously, the trial will enroll 326 patients with adaptive trial design and OS as a primary endpoint. The trial is expected to complete enrollment with interim data anticipated in 2H:15. Patients who are on quizartinib before the transplant are eligible to re-initiate quizartinib treatment after transplant, which may increase duration of remission and extend Overall Survival (OS) benefit. Management noted that while post-transplant maintenance is optional, physicians will likely implement it as a result of upcoming data to be presented in 2014 AMBI also has chemo combinations studies in preparation for potential expansion to the first-line setting.

• **Multiple data presentations at ASCO and EHA.** As previously announced, final results from the Phase II trial of quizartinib in FLT3-ITD positive R/R AML patients will be presented on June 2nd. At the same session, results from a trial of quizartinib bridge-to-transplant study will also be presented. The company also reported that two abstracts were accepted for presentation at the European Hematology Association (EHA; June 12-15). The abstracts from ASCO and ASH will be available online on May 14th and 21st, respectively.

• **Model update.** AMBI reported $0.032M in revenue and ($9.0M) in net loss, vs. our estimate of $3.5M and ($7.5M), respectively. The company ended the quarter with $61.9M 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 $14M to $0.13M and EPS estimate from ($1.62) to ($2.21).

Key Stats:	**(NASDAQ:AMBI)**
S&P 600 Health Care Index:	**1,212.60**
Price:	**$6.58**
Price Target:	$14.00
Methodology:	Prob. weighted NPV, 10% discount rate
52 Week High:	$21.44
52 Week Low:	$5.75
Shares Outstanding (mil):	17.9
Market Capitalization (mil):	$117.8
Book Value/Share:	$(0.46)
Cash Per Share:	$3.45
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	$6.6	$11.5	$7.7	$1.3	$27.1	($3,019.30)	$0.45	($0.34)	($0.11)	($1.19)	NM
2014E - New	**0.0A**	**0.0**	**0.0**	**0.0**	**$0.1**	**($0.50)**	**($0.58)**	**($0.57)**	**($0.57)**	**($2.21)**	**NM**
2014E - Old	$3.5	$3.5	$3.5	$3.5	$14.0	($0.41)	($0.41)	($0.41)	($0.40)	($1.62)	NM
2015E - New	**--**	**--**	**--**	**--**	**0.0**	**--**	**--**	**--**	**--**	**($1.83)**	**NM**
2015E - Old	--	--	--	--	$12.0	--	--	--	--	($1.45)	NM

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

Please refer to pages 6 - 9 for Important Disclosures, Price Charts and Analyst Certification.

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.

AMBI Upcoming Catalysts

Compound	Timing	Event
Quizartinib (FLT3i)	May 14, 2014	ASCO abstracts available
	May 21, 2014	EHA abstracts available
	May 30-June 3, 2014	2 poster presentations at ASCO
	June 12-15, 2014	2 presentations at EHA
	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 presented at ASH 2013.
R/R AML with Flt3-ITD	Phase III	Phase III in R/R AML with Flt3-ITD initiated in early '14.
Front line AML	Phase I	Frontline in combination with chemotherapy 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 11%, 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 ($K, except EPS)	**2012A**	**2013A**	**Mar-14A**	**Jun-14E**	**Sep-14E**	**Dec-14E**	**2014E**	**2015E**	**2016E**	**2017E**	**2018E**
Collaboration agreements	17,633	27,093	32	32	32	32	128	0			
Quizartinib sales									2,020	17,752	41,045
Total revenue	**17,633**	**27,093**	**32**	**32**	**32**	**32**	**128**	**0**	**2,020**	**17,752**	**41,045**
COGS									162	1,420	3,284
% of revenue									*8%*	*8%*	*8%*
R&D	36,731	26,284	6,257	7,500	7,500	7,500	28,757	29,620	30,508	31,424	32,366
SG&A	6,550	10,342	3,308	3,000	3,000	3,000	12,308	14,770	25,000	30,179	32,836
% of revenue										*170%*	*80%*
gain on sale of kinase profiling services	(2,497)	(2,500)	0	0	0	0	0	0	0	0	
Total operating expenses	40,784	34,126	9,565	10,500	10,500	10,500	41,065	44,389	55,670	63,023	68,486
Net income (loss) from operations	(23,151)	(7,033)	(9,533)	(10,468)	(10,468)	(10,468)	(40,937)	(44,389)	(53,650)	(45,270)	(27,441)
Interest expenses	(1,737)	(323)	0	0	0	0	0	0			
Other income	29	13	8	0	0	0	8	0			
Change in fair value of derivative liabilies	(2,291)	(3,942)	528	0	0	0	528	0			
Total other income (expenses)	(3,999)	(4,252)	536	0	0	0	536	0	0	0	0
Net income (loss) before income taxes	(27,150)	(11,285)	(8,997)	(10,468)	(10,468)	(10,468)	(40,401)	(44,389)	(53,650)	(45,270)	(27,441)
Provision (benefit) for income taxes	(121)	(29)	1	0	0	0	0	0			
Tax rate											
Net income (loss)	(27,029)	(11,256)	(8,998)	(10,468)	(10,468)	(10,468)	(40,401)	(44,389)	(53,650)	(45,270)	(27,441)
Non-controlling interest	382	61	0	0	0	0	200	200			
Net income (loss) attributable to AMBI	(26,647)	(11,195)	(8,998)	(10,468)	(10,468)	(10,468)	(40,201)	(44,189)	(53,650)	(45,270)	(27,441)
Other comprehensive income											
Accretion to redemption value of reddemable convertible preferred stock	(3,161)	(3,634)	0	0	0	0	0	0			
Change in fair value of redeemable non-controlling interest	(854)	1,747	0	0	0	0	0	0			
Net income to preferred stockholders			0	0	0	0	0				
Net loss to common stockholders	**(30,662)**	**(13,082)**	**(8,998)**	**(10,468)**	**(10,468)**	**(10,468)**	**(40,201)**	**(44,189)**	**(53,650)**	**(45,270)**	**(27,441)**
Foreign currency translation		(373)	(155)	0	0	0	(155)				
Net loss per share	**(16,591.99)**	**(1.19)**	**(0.50)**	**(0.58)**	**(0.57)**	**(0.57)**	**(2.21)**	**(1.83)**	**(1.77)**	**(1.42)**	**(0.75)**
Basic shares	2	11,024	17,937	18,117	18,298	18,481	18,208	24,119	30,325	31,841	36,433
Dilutive shares		17,479	21,800	22,454	23,128	23,590	22,743	28,880	35,324	37,090	41,945

Source: Company Reports and Leerink Partners

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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 $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 11%, which we believe is appropriate given probability-weighted sales projection.

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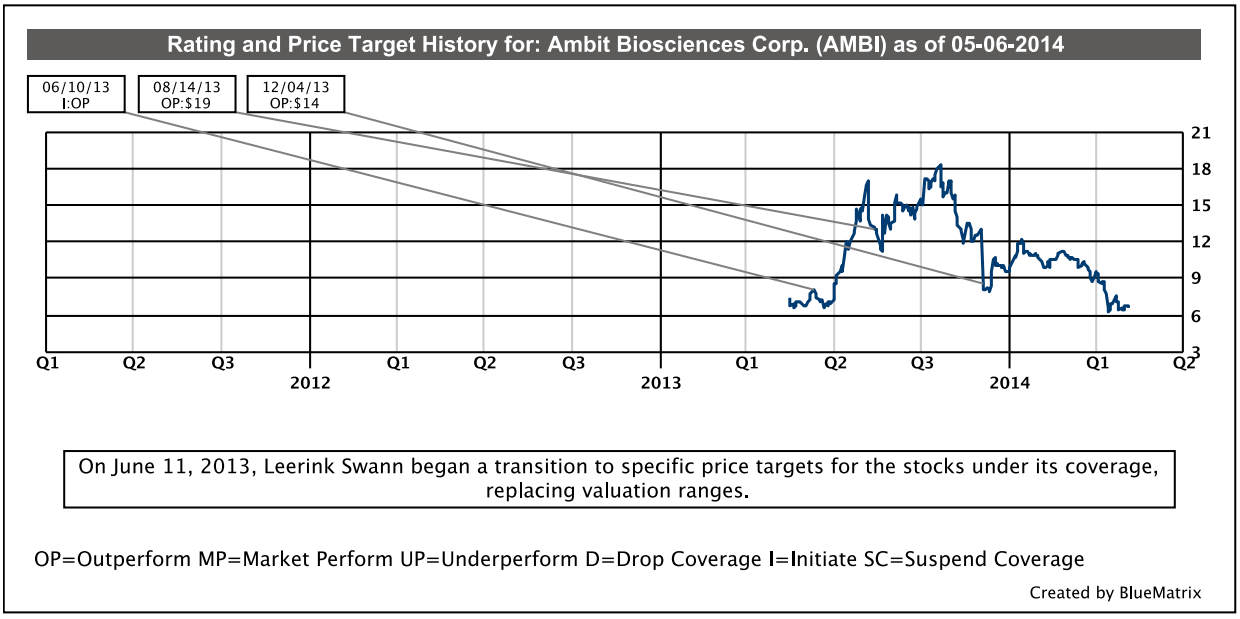

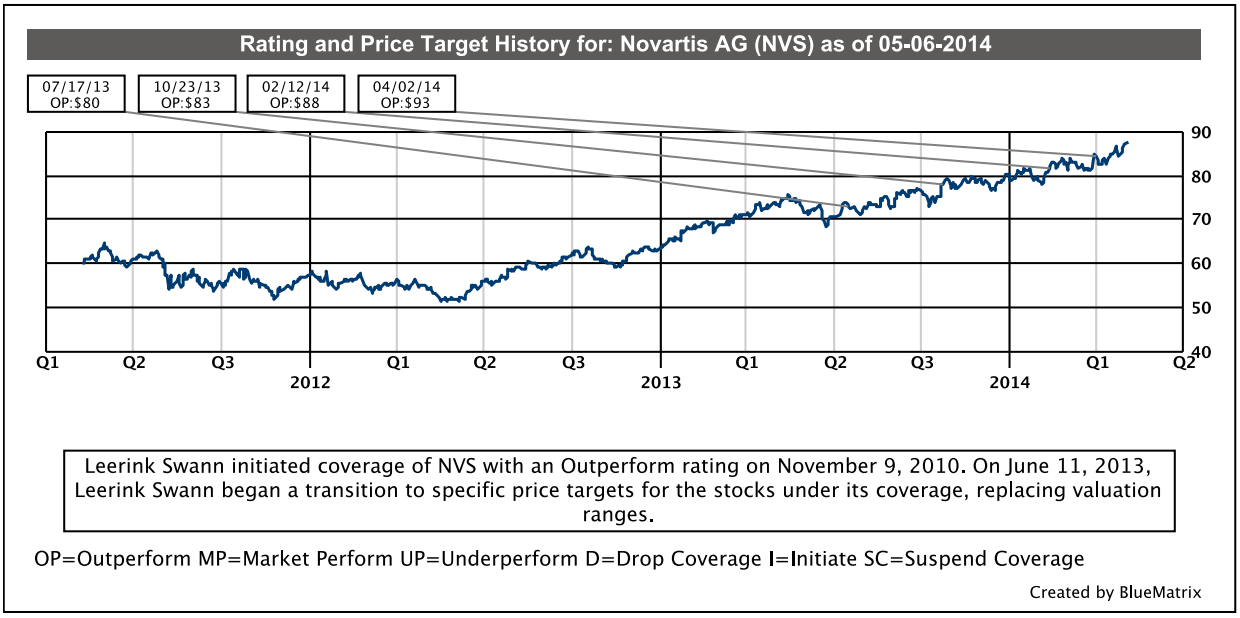
Rating and Price Target History for: Novartis AG (NVS) as of 05-06-2014
07/17/13 OP:$80
10/23/13 OP:$83
02/12/14 OP:$88
04/02/14 OP:$93
90
80
70
60
50
40
Q1
Q2
Q3
2012
Q1
Q2
Q3
2013
Q1
Q2
Q3
2014
Q1
Q2
Leerink Swann initiated coverage of NVS with an Outperform rating on November 9, 2010. On June 11, 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 03/31/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	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.

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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 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.

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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