December 4, 2013

OUTPERFORM

Reason for report:

COMPANY UPDATE

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LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

AMBIT BIOSCIENCE

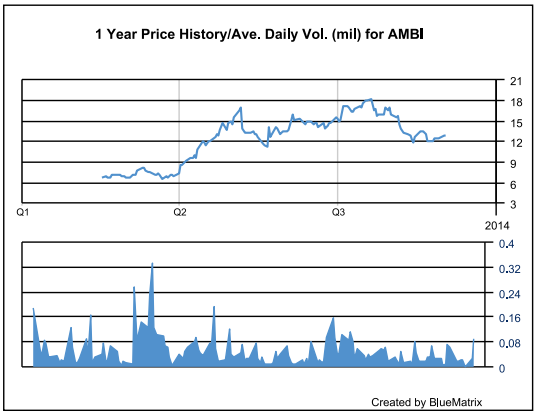
Near-Term Upside Scenario Removed, Long-Term Potential Remains

• **Bottom Line:** AMBI announced last night that following the end of Phase II meeting with the FDA, the company does not plan to file for an accelerated approval for quizartinib (FLT3 inhibitor) in acute myeloid leukemia (AML) based on Phase II/IIb data with surrogate endpoint of complete remission with incomplete hematologic recovery (CRi). Although the prior FDA meeting appeared to give some hope about the possibility of filing on Phase II data, whether CRi could be a surrogate endpoint for AML has been a highly controversial issue among MEDACorp key opinion leaders (KOLs) we spoke to and in our most recent discussions KOLs were cautious (LINK). The stock price going into the FDA meeting also appears to reflect reduced expectations. The decision removes a source of near-term upside for AMBI, although in the long-term, a controlled Phase III study would be required regardless, for a full approval. We note the upcoming Phase III readout for another FLT3 inhibitor midostaurin (NVS [OP]) could potentially help sentiment. We remove our 50% probability of accelerated approval and lower our valuation from $19 to $14 for AMBI.

• **A controlled Phase III will be required for approval, dosing still under discussion with the FDA.** The FDA remains unconvinced that CRi is an approvable endpoint, although there have been some evidence suggesting CRi is associated with certain degree of improvement in overall survival (ASH 2013, #2654). As noted previously, the Phase III trial will enroll 326 patients randomized at 2:1 with a single event-driven interim analysis and overall survival (OS) as primary endpoint. Following the end of Phase II meeting, AMBI will discuss with the FDA finalization of the Phase III trial design including dosing regimen. Management previously noted that 60mg would likely be the proposed dose.

• **Midostaurin Phase III outcome has read through to quizartinib.** Midostaurin, a less potent FLT3 inhibitor, showed a modest FLT3 inhibition during intensive induction therapy (ASH 2013, #1283, AMLSG16-10 trial). Although the complete response (CR) rate was promising (78.5%), there were no differences in FLT3 inhibition. Previously, midostaurin monotherapy in AML or MDS showed 71% bone marrow blasts reduction in FLT3 mutants vs. 42% in wild type patients, suggesting activity in both mutant and wild type patients (Fischer et al, JCO 2010, 28:4339). NVS started a Phase III trial with midostaurin in combination with cytarabine and daunorubicin and data readout is expected in July, 2014 (according to ClinicalTrial.gov) but could potentially be soon. Positive midostaurin Phase III outcome could provide clinical proof-of-concept for targeting FLT3. A negative midosaturin study would not necessarily mean that quizartinib Phase III would be negative as it is generally agreed that quarzartinib is a far more potent FLT3 inhibitor.

Key Stats:	**(NASDAQ:AMBI)**
S&P 600 Health Care Index:	**1,282.02**
Price:	**$12.80**
Price Target:	$14.00 from $19.00
Methodology:	probability-weighted NPV, 10% discount rate
52 Week High:	$21.44
52 Week Low:	$6.22
Shares Outstanding (mil):	17.9
Market Capitalization (mil):	$229.1
Book Value/Share:	$(0.46)
Cash Per Share:	$4.32
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	--	--	--	--	$17.6	--	--	--	--	($16,592.00)	NM
2013E	$6.6A	$11.5A	$7.7A	$2.0	$27.8	($3,019.30)A	$0.45A	($0.34)A	($0.40)	($1.48)	NM
2014E	--	--	--	--	$15.0	--	--	--	--	($1.58)	NM

Source: Company Information and Leerink Swann LLC Research
Revenues in millions.
GAAP EPS. Estimates reflect 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)	Dec 7-10, ASH 2013	Four abstracts including full Phase IIb data of 30 and 60mg dose from 76 pts in R/R AML with Flt3-ITD
	Early '14	Phase III initiation in R/R AML with Flt3-ITD
AC708 (CSF1Ri)	4Q:13	IND-enabling studies
	2Q:14	IND submission

Source: Company Reports and Leerink Swann

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 Swann

Valuation

We are lowering our valuation from $19 to $14 for AMBI following removal of probability of 2015 launch. We previously modeled 50% probability for a quizartinib launch in early 2015 and 50% probability for launch in late 2016 in the U.S, followed by a one-year delay in the EU and another year delay in Japan. Our new valuation 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 ($K, except EPS)	2011A	2012A	Mar-13A	Jun-13A	Sep-13A	Dec-13E	2013E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements	23,843	17,633	6,592	11,547	7,678	2,000	27,817	15,000	12,000			
Quizartinib sales										2,020	17,752	41,045
Total revenue	23,843	17,633	6,592	11,547	7,678	2,000	27,817	15,000	12,000	2,020	17,752	41,045
COGS										162	1,420	3,284
% of revenue										*8%*	*8%*	*8%*
R&D	50,705	36,731	9,005	6,664	4,484	6,000	26,153	32,691	33,672	34,682	35,723	36,794
SG&A	8,905	6,550	1,776	2,197	3,076	3,230	10,279	11,307	12,437	25,000	30,179	32,836
% of revenue											*170%*	*80%*
gain on sale of kinase profiling services	(2,108)	(2,497)	0	0	(2,500)	0	(2,500)	0	0	0	0	
Total operating expenses	57,502	40,784	10,781	8,861	5,060	9,230	33,932	43,998	46,109	59,844	67,322	72,914
Net income (loss) from operations	(33,659)	(23,151)	(4,189)	2,686	2,618	(7,230)	(6,115)	(28,998)	(34,109)	(57,823)	(49,569)	(31,869)
Interest expenses	(4,502)	(1,737)	(162)	2,474	0	0	2,312	0	0			
Other income	1,538	29	7	0	(8,687)	0	(8,680)	0	0			
Change in fair value of derivative liabilities	(795)	(2,291)	(3,957)	0	0	0	(3,957)	0	0			
Total other income (expenses)	(3,759)	(3,999)	(4,112)	2,474	(8,687)	0	(10,325)	0	0	0	0	0
Net income (loss) before income taxes	(37,418)	(27,150)	(8,301)	5,160	(6,069)	(7,230)	(16,440)	(28,998)	(34,109)	(57,823)	(49,569)	(31,869)
Other comprehensive income				3			3					
Provision (benefit) for income taxes	0	(121)	1	0	0	0	1	0	0			
Tax rate												
Net income (loss)	(37,418)	(27,029)	(8,302)	5,163	(6,069)	(7,230)	(16,438)	(28,998)	(34,109)	(57,823)	(49,569)	(31,869)
Non-controlling interest	(213)	382	73	0	0	50	123	200	200			
Net income (loss) attributable to AMBI	(37,631)	(26,647)	(8,229)	5,163	(6,069)	(7,180)	(16,315)	(28,798)	(33,909)	(57,823)	(49,569)	(31,869)
Accretion to redemption value of reddemable convertible preferred stock	(2,000)	(3,161)	(2,319)	(1,315)	0	0	(3,634)	0	0			
Change in fair value of redeemable non-controlling interest	4,477	(854)	(1,499)	3,246	0	0	1,747	0	0			
Net income allocated to common stockholders				(3,457)			(3,457)					
Net loss to common stockholders	(35,154)	(30,662)	(12,047)	3,637	(6,069)	(7,180)	(21,659)	(28,798)	(33,909)	(57,823)	(49,569)	(31,869)
Net loss per share	**(25,886.60)**	**(16,591.99)**	**(3,019.30)**	**0.45**	**(0.34)**	**(0.40)**	**(1.48)**	**(1.58)**	**(1.40)**	**(1.90)**	**(1.56)**	**(0.87)**
Basic shares	1	2	4	8,055	17,877	18,056	14,663	18,236	24,148	30,356	31,873	36,467
Dilutive shares				9,752	21,208	21,420	17,460	21,634	27,716	34,102	35,807	40,597

Source: Company Reports and Leerink Swann

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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.

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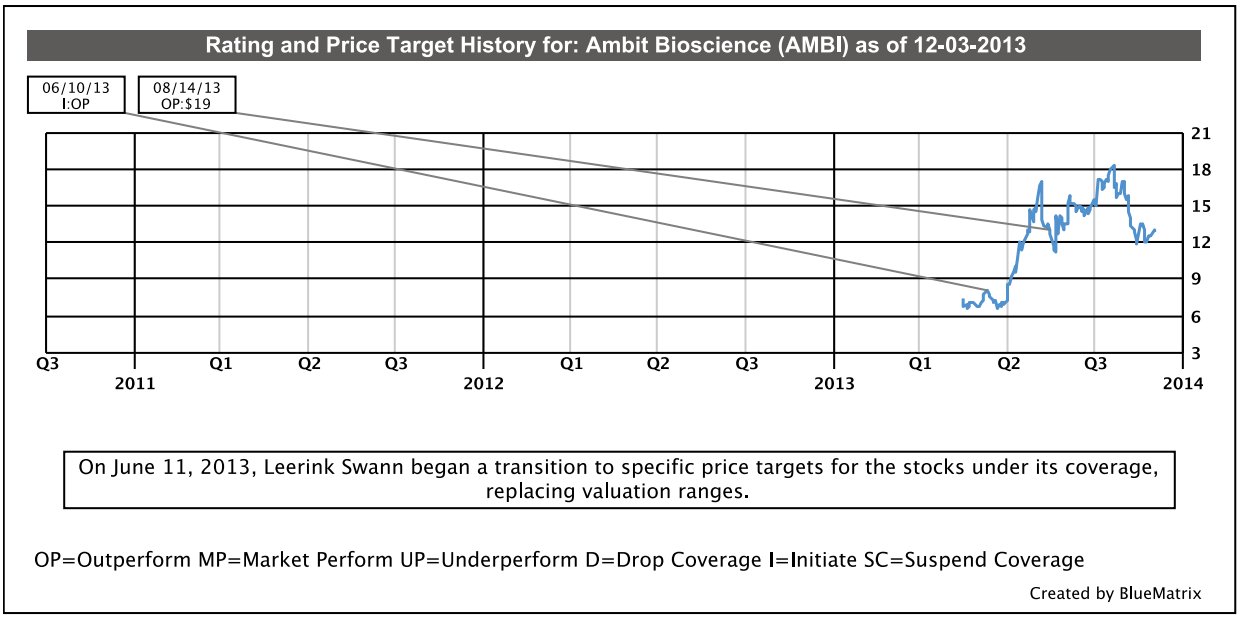

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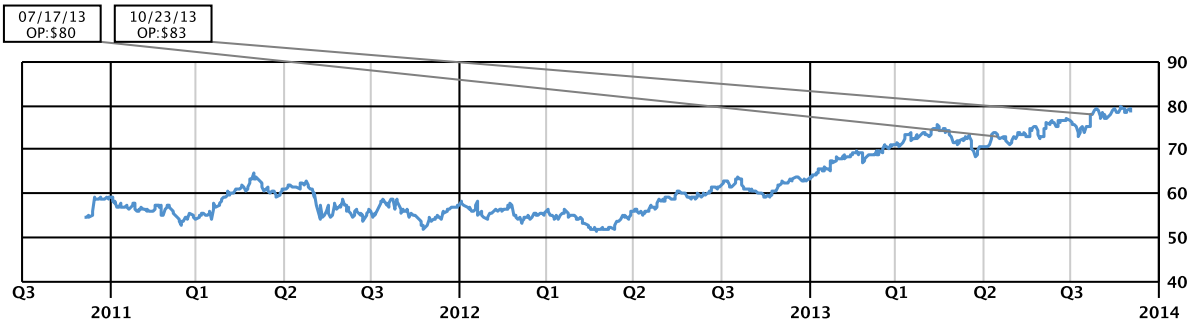
Rating and Price Target History for: Novartis AG (NVS) as of 12-03-2013



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

Leerink Swann LLC makes a market in Ambit Bioscience.

Leerink Swann 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 Swann LLC has acted as the manager for a public offering of Ambit Bioscience in the past 12 months.

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