August 14, 2013

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

AMBIT BIOSCIENCE

New Developments Enhance Likelihood of Early NDA Submission

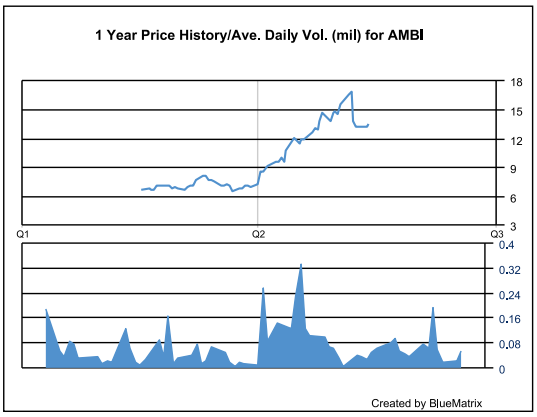
• **Bottom Line:** On its 2Q:13 call, AMBI management highlighted several important positive developments for advancing quizartinib, an Flt3 inhibitor in acute myeloid leukemia (AML) patients with positive Flt3-ITD mutation. Our takeaways include: 1) the FDA has noted that the overall clinical benefit of quizartinib will be a "review issue", which would appear to indicate a willingness of the agency to review the filing based on current data, pending additional requested information; 2) independent analyses supporting CRc (composite complete response) and CRi (incomplete hematologic recovery) as surrogate endpoints are now available to AMBI and will be presented at ASH; 3) full data on lower doses (30 mg/60 mg) of quizartinib are consistent with earlier findings; 4) based on additional information requested by the FDA, the end of Phase II (EOP2) meeting is slightly delayed (Nov. vs. prior Sept.), which we do not view to be significant. We believe the updates increase the chance of a NDA filing based on Phase II/IIb data. We are incorporating a probability-weighted (50%) scenario of early approval in 2015 and increasing our valuation for AMBI from $14 to $19.

• **AMBI appears well positioned to address FDA request of additional information for EOP2 meeting.** AMBI had a "Type C" meeting with the FDA in June, and the FDA requested additional data for the EOP2 meeting -- primarily 1) further analysis of the Phase II and Phase IIb data with sufficient data to support proposed dose and label, 2) historical data supporting CRi as an endpoint for predicting clinical benefit, which entails comparing survival of patients who achieved CRi vs. those who did not. Management commented that, although such data have not been published, there are now multiple analyses from at least two institutions. The company has access to these data, which are expected to be presented at the ASH 2013 meeting (Dec. 7-10).

• **Although a final decision has not been made, management suggested 60mg as the proposed dose, full data to be presented at ASH 2013.** Although final dose for the Phase III trial and the NDA submission will be decided after the EOP2 meeting with the FDA, based on the existing efficacy/safety data, management suggested 60mg as the proposed dose. Full Phase IIb data set with both 30mg and 60 mg doses in 76 relapsed/refractory AML patients with Flt3-ITD will be presented at ASH.

• **On track to initiate quizartinib Phase III trial in early 2014.** The outcome of the EOP2 meeting is unlikely to affect the quizartinib Phase III clinical trial plan, and management is on track to initiate the Phase III trial in early 2014.

Key Stats:	**(NASDAQ:AMBI)**
S&P 600 Health Care Index:	**1,118.58**
Price:	**$13.50**
Price Target:	$19.00
Methodology:	NPV analysis
52 Week High:	$17.11
52 Week Low:	$6.22
Shares Outstanding (mil):	17.9
Market Capitalization (mil):	$241.7
Book Value/Share:	$(0.46)
Cash Per Share:	$4.77
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 - New	**$6.6A**	**$11.5A**	**$10.0**	**$2.0**	**$30.1**	**($3,019.30)A**	**$0.45A**	**$0.03**	**($0.57)**	**($0.99)**	**NM**
2013E - Old	$6.6A	$6.5	$5.0	$3.0	$21.1	($3,019.30)A	($0.27)	($0.38)	($0.52)	($1.85)	NM
2014E - New	**--**	**--**	**--**	**--**	**$15.0**	**--**	**--**	**--**	**--**	**($1.66)**	**NM**
2014E - Old	--	--	--	--	$15.0	--	--	--	--	($1.77)	NM

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

Please refer to pages 6 - 8 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. In addition, KOL feedback indicates that CRi, which represents the majority of responses seen on quizartinib, does enable stem cell transplant which is viewed as the only hope for relapsed/refractory AML patients. Therefore CRi confers a clinically meaningful benefit. While we advocate valuing AMBI based on the assumption of approval after the completion of Phase III, we believe the possibility of filing based on Phase II data exists and there is a realistic chance of approval if the application gets to an FDA advisory panel review. Although the FDA previously did not support filing for quizartinib based on early Phase II data, there has been at least one case (Marqibo) in which accelerated approval was obtained based on CRi, and data appeared far more limited, in our view. At the current valuation, we believe accelerated approval would be all upside.

Multiple data potentially at ASH. Besides full Phase IIb data and historical data supporting CRc and CRi, additional data include Phase I top-line data for quizartinib/chemo combination in younger newly diagnosed (ND) AML patients, full data from Phase Ib/II investigator-sponsored trial in older ND AML patients, and 3) final data from TACL Phase I pediatric trial in less than 21 years old relapsed ALL or AML patients.

Model Update. Yesterday after the market close, AMBI reported $11.5M in 2Q:13 revenue, higher than our estimate of $6.5M, mainly due to an increase in license fee amortization related to the Astellas collaboration. Net income was $5.2M vs. our estimate of ($4.8M). Basic EPS was $0.45 vs. our estimate of ($0.27). The company ended the quarter with $85M in cash, sufficient to support operations through the quizartinib Phase III trial. We are updating our model to reflect these changes, and our 2013 revenue projection changes from $21M to $30M and our EPS estimate changes from ($1.85) to ($0.99).

AMBI Upcoming Catalysts

Compound	Timing	Event
Quizartinib (FLT3i)	Nov, '13	End of Phase II meeting with FDA
	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
	YE:15	Phase III data
AC708 (CSF1Ri)	2H:13	Initiating 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 increasing our valuation for AMBI from $14 to $19 by including a scenario of early NDA submission based on Phase II data. We now model 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 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. Our new valuation is $19 based on probability-weighted NPV valuation methodology. We use a discount rate of 10%, which we believe is appropriate given probability-weighted sales projection. Our previous valuation of $14 was based on filing after Phase III data, using the same estimates.

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.
- Regulatory risk -- achieving accelerated approval based on current Phase II data, which represents the upside scenario, faces considerable uncertainty given that the FDA previously appeared unsupportive of such filing based on early Phase II data.
- 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 (Base Case)	2011A	2012A	Mar-13A	Jun-13A	Sep-13E	Dec-13E	2013E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements	23,843	17,633	6,592	11,547	10,000	2,000	30,139	15,000	12,000			
Quizartinib sales										2,020	17,752	41,045
Total revenue	23,843	17,633	6,592	11,547	10,000	2,000	30,139	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	6,997	10,000	32,666	35,933	37,011	38,121	39,265	40,443
G&A	8,905	6,550	1,776	2,197	2,307	2,422	8,702	9,572	10,529	25,000	30,179	32,836
% of revenue											*170%*	*80%*
gain on sale of kinase profiling services	(2,108)	(2,497)	0	0	0	0	0	0	0	0	0	
Total operating expenses	57,502	40,784	10,781	8,861	9,304	12,422	41,368	45,505	47,540	63,283	70,864	76,563
Net income (loss) from operations	(33,659)	(23,151)	(4,189)	2,686	696	(10,422)	(11,229)	(30,505)	(35,540)	(61,262)	(53,112)	(35,517)
Interest expenses	(4,502)	(1,737)	(162)	2,474	0	0	2,312	0	0			
Other income	1,538	29	7	0	0	0	7	0	0			
Change in fair value of derivative liabilies	(795)	(2,291)	(3,957)	0	0	0	(3,957)	0	0			
Total other income (expenses)	(3,759)	(3,999)	(4,112)	2,474	0	0	(1,638)	0	0	0	0	0
Net income (loss) before income taxes	(37,418)	(27,150)	(8,301)	5,160	696	(10,422)	(12,867)	(30,505)	(35,540)	(61,262)	(53,112)	(35,517)
Other comprehensive income				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	696	(10,422)	(12,868)	(30,505)	(35,540)	(61,262)	(53,112)	(35,517)
Non-controlling interest	(213)	382	73	0	50	50	173	200	200			
Net income (loss) attributable to AMBI	(37,631)	(26,647)	(8,229)	5,163	746	(10,372)	(12,695)	(30,305)	(35,340)	(61,262)	(53,112)	(35,517)
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)								
Net loss to common stockholders	(35,154)	(30,662)	(12,047)	3,637	746	(10,372)	(14,582)	(30,305)	(35,340)	(61,262)	(53,112)	(35,517)
Net loss per share	**(25,886.60)**	**(16,591.99)**	**(3,019.30)**	**0.45**	**0.03**	**(0.57)**	**(0.99)**	**(1.66)**	**(1.46)**	**(2.02)**	**(1.67)**	**(0.97)**
Basic shares	1	2	4	8,055	17,890	18,069	14,672	18,250	24,162	30,370	31,889	36,483
Dilutive shares				9,752	21,693	21,910	17,785	22,129	28,235	34,647	36,380	41,198

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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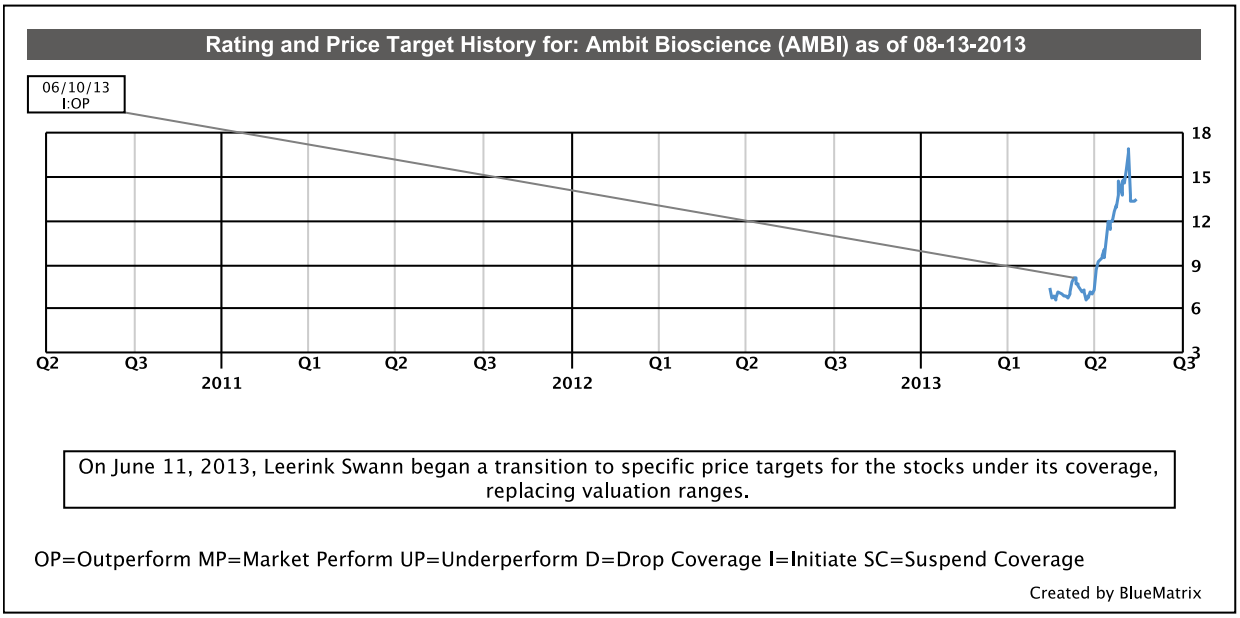

Distribution of Ratings/Investment Banking Services (IB) as of 06/30/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	103	62.80	30	29.00
HOLD [MP]	61	37.20	2	3.00
SELL [UP]	0	0.00	0	0.00

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 Swann LLC makes a market in Ambit Bioscience.

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