November 8, 2013

OUTPERFORM

Reason for report:

EARNINGS

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HEALTHCARE EQUITY RESEARCH

AMBIT BIOSCIENCE

3Q:13 Highlights On Track Development, Reaffirms EOP2 FDA Meeting

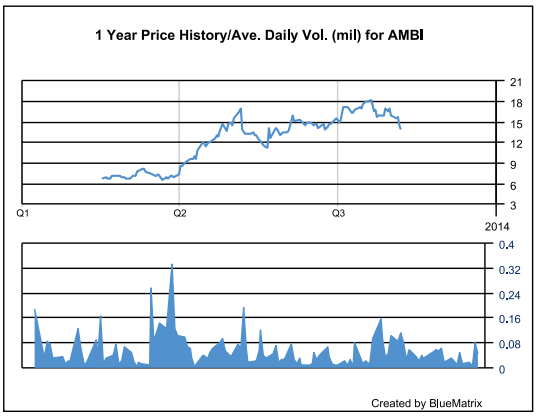
• **Bottom Line:** Following ASH abstract release yesterday morning, AMBI highlighted on the earnings call new data for quizartinib/chemo combination therapy in both elderly newly diagnosed acute myeloid leukemia (AML) patients and young relapsed/refractory AML patients. As anticipated, full Phase II data with all doses will be presented at ASH. The company reaffirmed the timing for end of Phase II (EOP2) meeting with the FDA (end of November) and for initiation of the Phase III trial (1Q:14) with slightly more clarity on the trial design. Key catalysts in the near-term remain the EOP2 meeting and the potential filing for accelerated approval based on Phase II data. Although opinions are still mixed, we increasingly hear a supportive view from MEDACorp key opinion leaders. Data from one poster (#2654) at ASH adds to supportive evidence of survival benefit from CRi (complete response with incomplete blood count recovery) and CRi as a surrogate endpoint which in turn supports quizartinib filing in our opinion.

• **ASH poster shows CRi is correlated with Overall Survival (OS) benefit, adds to previous published data.** AMBI has collected public and private information on CRi benefit in overall survival to prepare for the EOP2 FDA meeting. According to management, AMBI has the right to use non-public information as supportive evidence but it is up to investigators to disclose the data. So far, one poster (#2654 by MD Anderson (see graph on page 2) at ASH showed supportive evidence where pooled CRi/PR/MLF(morphologically leukemia free) after salvage therapy was associated with improved survival vs. non-responders. These data are additive to previously published paper by Walter et al (JCO 2010) that shows CRi is associated with OS improvement relative to non-responders, although the magnitude of OS benefit is not as robust vs. patients who achieved CR.

• **More clarity on Phase III trial, final design after EOP2 with the FDA.** Consistent with prior proposal, Phase III trial will enroll 326 patients randomized at 2:1, with a single event-driven interim analysis and OS as primary endpoint. Statistical powering will be finalized after discussion with the FDA. Enrollment will start in early 2014 with an estimated ~17 months (till the end of 2015) for accrual. Although not final, as noted before, 60mg is likely the proposed dose for the Phase III trial.

• **Multiple data at ASH as stated before.** Besides full Phase IIb data, additional data include Phase I top-line data for quizartinib/chemo combination in old (>60 years old) and adult newly diagnosed (ND) AML patients, and final data from Phase I pediatric trial (<21 years old) in relapsed/refractory ALL or AML patients.

Key Stats:	**(NASDAQ:AMBI)**
S&P 600 Health Care Index:	**1,181.68**
Price:	**$14.00**
Price Target:	$19.00
Methodology:	DCF with 10% discount rate
52 Week High:	$21.44
52 Week Low:	$6.22
Shares Outstanding (mil):	17.9
Market Capitalization (mil):	$250.6
Book Value/Share:	$(0.46)
Cash Per Share:	$4.37
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**	**$7.7A**	**$2.0**	**$27.8**	**($3,019.30)A**	**$0.45A**	**($0.34)A**	**($0.40)**	**($1.48)**	**NM**
2013E - Old	$6.6A	$11.5A	$10.0	$2.0	$30.1	($3,019.30)A	$0.45A	$0.03	($0.57)	($0.99)	NM
2014E - New	**--**	**--**	**--**	**--**	**$15.0**	**--**	**--**	**--**	**--**	**($1.58)**	**NM**
2014E - Old	--	--	--	--	$15.0	--	--	--	--	($1.66)	NM

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

Please refer to pages 7 - 9 for Important Disclosures, Price Charts and Analyst Certification.

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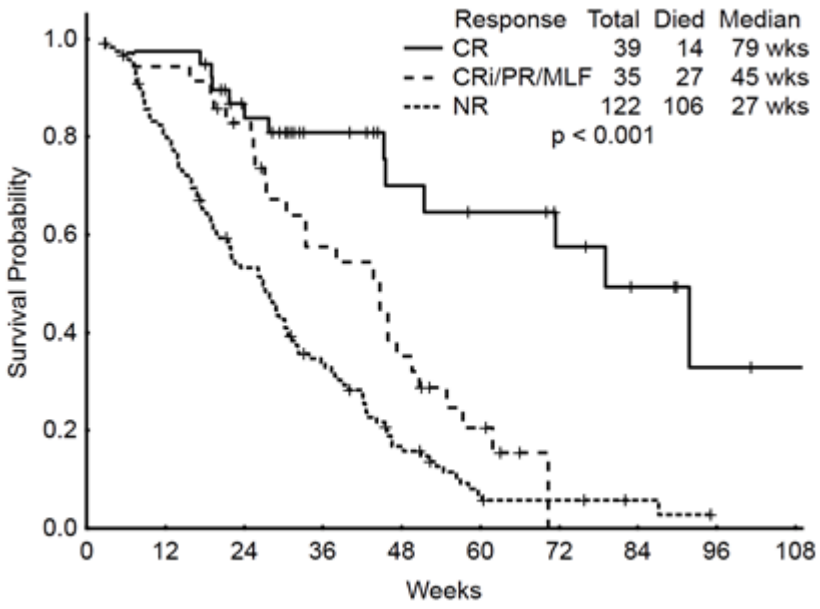
Investment Thesis

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.

CRi Showed Improved Survival vs. Non-Responders, But Reduced Benefit vs. CR



Source: Shah et al, #2654, ASH 2013

Model Update. For 3Q:13, AMBI reported $7.7M in revenue and ($0.34) in EPS. The company ended the quarter with $79M 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 $30M to $28M and our EPS estimate changes from ($0.99) to ($0.34).

AMBI Upcoming Catalysts

Compound	Timing	Event
Quizartinib (FLT3i)	Late 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
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

Our $19 is derived from probability-weighted NPV valuation methodology. We 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. 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.
- 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 ($MM)	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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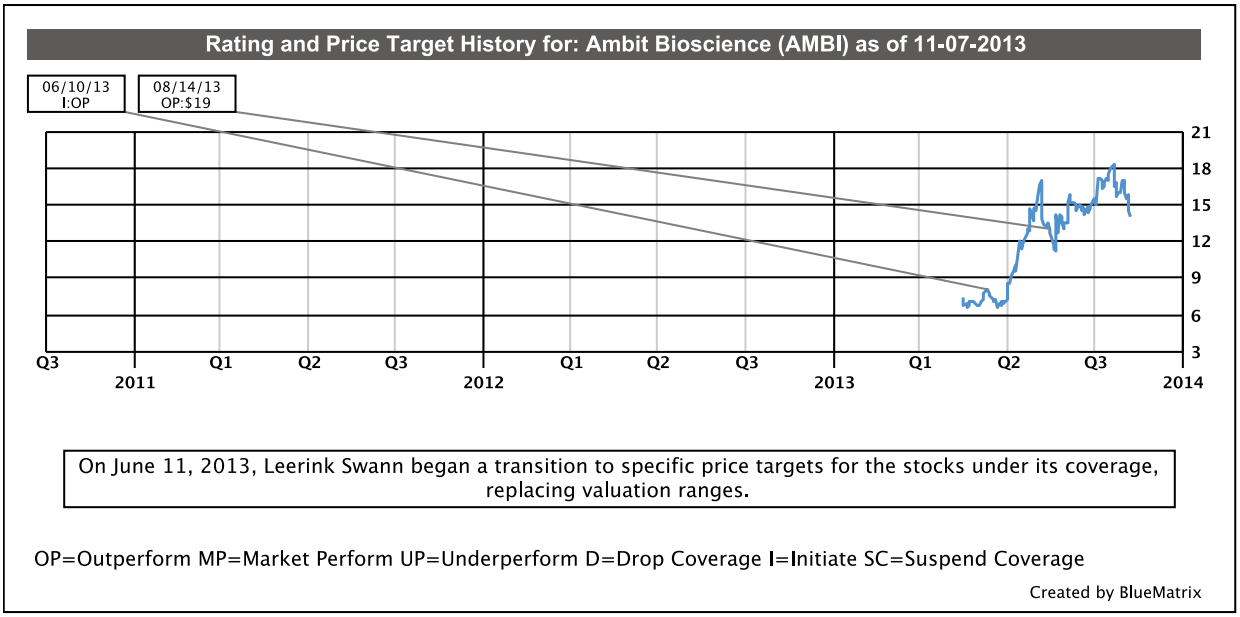

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	111	64.90	27	24.00
HOLD [MP]	60	35.10	0	0.00
SELL [UP]	0	0.00	0	0.00

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 Ambit Bioscience.

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