

bluebird bio

1Q Snapshot - LentiGlobin Clinical Update Coming Next Month at EHA

We are updating our BLUE model based on the company's 1Q results (there was no conference call). BLUE continues to make solid progress with its pipeline, and we are maintaining our OW rating. Importantly, we learned today that the company will have a clinical update sooner than anticipated; initial data from the B-thalassemia study is now anticipated at EHA in June (as opposed to late 2014). In our view, BLUE – with its gene therapy platform – is a potentially transformative and disruptive company that appears to be more than just a “big idea.” The company has already established promising proof-of-concept for its two lead gene therapy products, Lenti-D and LentiGlobin, and is going after orphan indications with a very high unmet medical need that could bolster the ultimate likelihood of success.

- **Upcoming events: clinical update next month at EHA.** An abstract highlighting preliminary clinical data from the ongoing Phase 1/2 HGB-205 Study of LentiGlobin for the treatment of beta-thalassemia major has been accepted for presentation at the European Hematology Association (EHA) Congress to be held in Milan, Italy from June 13-16. EHA abstracts will be available online next week (Wed, May 21). BLUE also expects to present additional Phase 1/2 data for LentiGlobin in beta-thalassemia (from the HGB-205 and Northstar studies) in late 2014. Finally, BLUE now has an active IND for LentiGlobin in the US for sickle cell disease (SCD); the US trial will be called HGB-206, and the co expects to transplant the first SCD patient with LentiGlobin in 2014 (in the HGB-205 or HGB-206 studies).
- **1Q results.** 1Q EPS was \$(0.44), slightly wider vs. consensus of \$(0.31). 1Q revs were \$6.3M, inline with consensus and JPMe of \$6.3M. 1Q OpEx of \$17.0M was slightly higher vs. consensus of ~\$14.4M and JPMe of \$15.4M. That said, BLUE is not yet an earnings story, in our view.
- **Minor estimate revisions; maintaining \$44 YE14 PT.** We have updated our model for 1Q results. Changes involved modest increases in OpEx but our YE14 PT remains \$44.
- **Balance sheet update.** BLUE ended 1Q with \$192M in cash (net cash used in operating activities was \$12M) and no debt. Given this solid cash position, BLUE should have sufficient capital to fund operations for several years.

bluebird bio, Inc. (BLUE;BLUE US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)
EPS reported (\$)			
Q1 (Mar)	-	-	(0.44)A
Q2 (Jun)	(2.13)	-	(0.46)
Q3 (Sep)	(0.26)	-	(0.46)
Q4 (Dec)	(0.33)	-	(0.45)
FY	(2.02)	(1.58)	(1.80)
Bloomberg EPS FY (\$)	-1.66	-	-1.39

Source: Company data, Bloomberg, J.P. Morgan estimates.

Overweight

BLUE, BLUE US

Price: \$19.42

Price Target: \$44.00

Biotechnology

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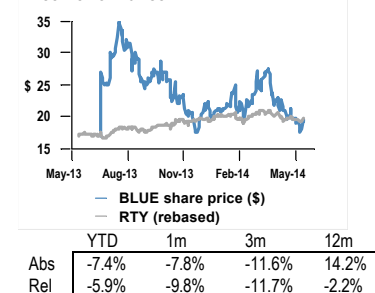
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Price Performance



Company Data

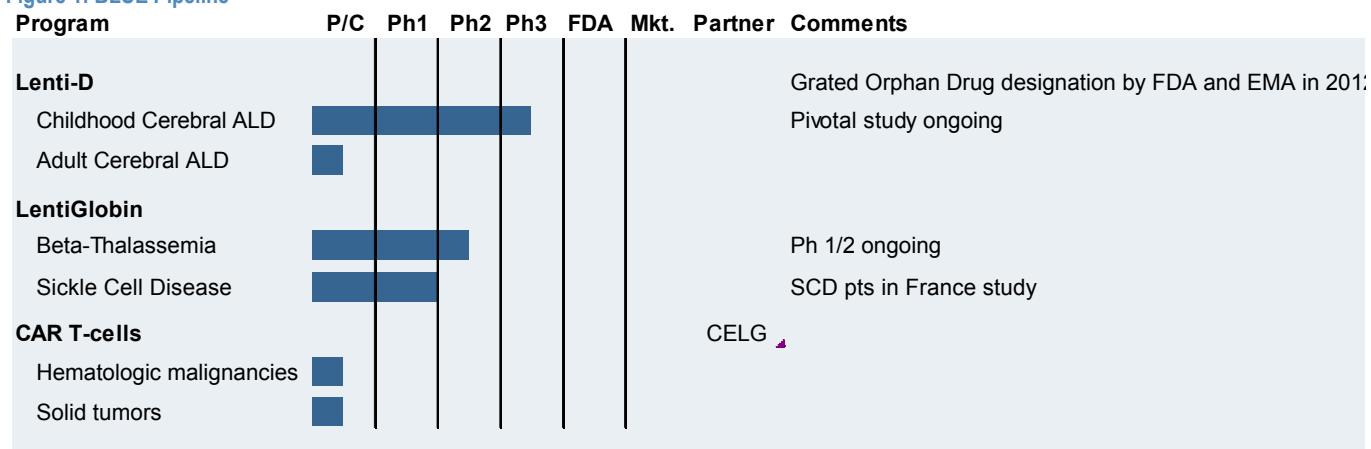
Price (\$)	19.42
Date Of Price	13 May 14
52-week Range (\$)	36.25-17.00
Market Cap (\$ mn)	469.96
Fiscal Year End	Dec
Shares O/S (mn)	24
Price Target (\$)	44.00
Price Target End Date	31-Dec-14

See page 6 for analyst certification and important disclosures.

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Pipeline

Figure 1: BLUE Pipeline



Source: Company reports and J.P. Morgan estimates.

Upcoming Events

Figure 2: BLUE Upcoming Events

Program	Event	Expected Timing	Significance
LentiGlobin	Preliminary data from HGB-205 Ph1/2 Study in beta-thalassemia major pts at EHA	June 13-16, 2014	Medium
	Transplant first SCD patient in HGB-205 or HGB-206 Studies	2014	Low
	Additional Phase 1/2 data for beta-thalassemia from HGB-205 and Northstar trials	Late 2014	High
Lenti-D	Complete enrollment in Starbeam Study	2015	Medium
CAR T-cells	Initiate Phase 1 trial	2016	Low-Medium

Source: Company reports and J.P. Morgan estimates.

Figure 3: BLUE Income Statement

BlueBird Bio Income Statement

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Fiscal Year Ends Dec 31	2012A	2013E	1Q14A	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Total Revenue	\$ 0.3	\$ 20.2	\$ 6.3	\$ 6.3	\$ 6.3	\$ 6.3	\$ 25.3	\$ 25.3	\$ 5.6	\$ 0.3	\$ 25.9
COGS	-	-	-	-	-	-	-	-	-	-	6.5
Gross Profit	0.1	20.2	6.3	6.3	6.3	6.3	25.3	25.3	5.6	0.3	19.5
R&D	17.2	31.0	11.5	12.0	12.1	12.1	47.7	50.8	52.0	57.5	58.5
Sales and Marketing	-	-	-	-	-	-	-	-	21.0	26.3	26.3
General and Administrative	6.8	14.1	5.5	5.6	5.7	5.8	22.6	26.0	27.3	28.7	30.1
Total Operating Expenses	\$ 24.1	\$ 45.1	\$ 17.0	\$ 17.6	\$ 17.8	\$ 17.9	\$ 70.3	\$ 76.8	\$ 100.3	\$ 112.5	\$ 121.4
Operating income	(23.7)	(24.9)	(10.7)	(11.3)	(11.5)	(11.6)	(45.0)	(51.4)	(94.7)	(112.1)	(95.4)
Other income, net	0.0	(0.4)	0.1	0.1	0.1	0.1	0.3	2.0	2.9	2.6	0.5
Pretax Income	(23.7)	(25.3)	(10.6)	(11.2)	(11.4)	(11.5)	(44.6)	(49.5)	(91.8)	(109.5)	(94.9)
Income Tax (benefit)	-	-	-	-	-	-	-	-	-	-	-
Net Income	\$ (23.7)	\$ (25.3)	\$ (10.6)	\$ (11.2)	\$ (11.4)	\$ (11.5)	\$ (44.6)	\$ (49.5)	\$ (91.8)	\$ (109.5)	\$ (94.9)
Net Loss applicable to common stockholders	(3.614)	(25.3)	(10.6)	(11.2)	(11.4)	(11.5)	(44.6)	(49.5)	(91.8)	(109.5)	(94.9)
Average shares Outstanding		12.6	24.1	24.5	24.9	25.3	24.7	26.3	32.0	34.0	36.0
EPS, Basic and Diluted		(2.02)	(0.44)	(0.46)	(0.46)	(0.45)	(1.80)	(1.88)	(2.87)	(3.22)	(2.64)
Margin Analysis:											
Gross margin	NM	100%	NM	NM	NM	NM	100%	100%	100%	NM	NM
Operating margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Cost Analysis:											
COGS as % of tot. prod. sales	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	25%
R&D as % of tot. revenue	5061.76%	153.62%	180.95%	189.42%	191.00%	191.00%	188.09%	200.28%	930.23%	16911.76%	225.53%
S&M as % of tot. revenue	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	375.67%	7720.59%	101.20%
G&A as % of tot. revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Year-over-year growth:											
Total revenue		5835.59%	462.11%	0.02%	-0.78%	0.00%	25.56%	0.00%	-77.94%	-93.92%	7529.08%
R&D Expense		80.14%	116.94%	65.59%	38.98%	23.91%	53.74%	6.48%	2.46%	10.58%	1.74%
S&M Expense		NM	138.38%	70.68%	48.59%	23.80%	NM	NM	NM	25.00%	0.00%
G&A Expense		106.34%					60.27%	15.00%	5.00%	5.00%	5.00%
Total operating expenses		87.60%	123.49%	67.17%	41.92%	23.88%	55.79%	9.22%	30.67%	12.08%	7.93%
Operating income		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net income		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
EPS		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Tax Rate		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM

Source: J.P. Morgan estimates, Company data.

Investment Thesis, Valuation and Risks

bluebird bio (Overweight; Price Target: \$44.00)

Investment Thesis

We have an OW rating on BLUE. In our view, BLUE – with its gene therapy platform – is one of the more potentially transformative and disruptive companies we've come across in some time. Importantly, BLUE has already established promising proof-of-concept for its two lead products, Lenti-D and LentiGlobin, and is going after orphan indications (such as CCALD and beta-thalassemia) with a very high unmet medical need that could bolster the ultimate probability of success.

Valuation

We have a YE14 price target of \$44 for BLUE. Our valuation methodology is based on a blended average of our risk-adjusted NPV model (33%), our proprietary scenario analysis (33%), and a DCF analysis (33%) and reflects a 50% probability of success for Lenti-D (~\$250M in peak sales) and 25% for LentiGlobin (~\$1B peak). We assign each valuation method a 15% discount rate, which we believe is appropriate given the probability-adjustments made to each development program.

BLUE Valuation Summary

BlueBird Bio : Valuation Summary			
Discount rate	15%		
Main value driver	Prob of approval	Peak sales est (avg. scenario)	Avg peak yr
CCALD	50%	\$ 260	2022
B-Thalassemia	25%	\$ 1,204	2023
Sickle Cell Disease	0%	\$ -	-
Valuation methodology	Value	Weighting	Adj. value/ share
P/E 2015	\$ -	0%	\$ -
Real options scenario analysis	\$ 43.14	33%	14.4
Risk adjusted NPV analysis	\$ 34.33	33%	11.4
DCF analysis	\$ 53.87	33%	18.0
Total			\$ 43.78
Catalyst/liquidity discount			0%
YE14 Valuation			\$ 44

Source: J.P. Morgan Estimates

Risks to Rating and Price Target

Downside risks to our OW recommendation include the standard issues that apply to the entire biotechnology industry, including development, regulatory, commercial, manufacturing, financing, and IP pitfalls. Other risks specific to BLUE include clinical trial risk with ongoing studies involving Lenti-D or LentiGlobin, regulatory uncertainty surrounding gene therapy, the company's ability to deliver gene therapies on a commercially viable scale, and competition within the gene therapy field.

bluebird bio: Summary of Financials

Income Statement - Annual	FY13A	FY14E	FY15E	FY16E	Income Statement - Quarterly	1Q14A	2Q14E	3Q14E	4Q14E
Revenues	20	25	25	-	Revenues	6A	6	6	6
Cost of products sold	0	0	0	-	Cost of products sold	0A	0	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(14)	(23)	(26)	-	SG&A	(6)A	(6)	(6)	(6)
R&D	(31)	(48)	(51)	-	R&D	(11)A	(12)	(12)	(12)
Operating income	(25)	(45)	(51)	-	Operating income	(11)A	(11)	(11)	(12)
EBITDA	(25)	(45)	(51)	-	EBITDA	(11)A	(11)	(11)	(12)
Net interest (income) / expense	0	0	2	-	Net interest (income) / expense	0A	0	0	0
Other income / (expense)	(0)	0	0	-	Other income / (expense)	0A	0	0	0
Income taxes	0	0	0	-	Income taxes	0A	0	0	0
Net income - GAAP	(25)	(45)	(49)	-	Net income - GAAP	(11)A	(11)	(11)	(11)
Net income - recurring	(25)	(45)	(49)	-	Net income - recurring	(11)A	(11)	(11)	(11)
Diluted shares outstanding	13	25	26	-	Diluted shares outstanding	24A	25	25	25
EPS - excluding non-recurring	(2.02)	(1.80)	(1.88)	-	EPS - excluding non-recurring	(0.44)A	(0.46)	(0.46)	(0.45)
EPS - recurring	(2.02)	(1.80)	(1.88)	-	EPS - recurring	(0.44)A	(0.46)	(0.46)	(0.45)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	238	207	170	-	Sales growth	5835.6%	25.6%	0.0%	-
Accounts receivable	0	0	0	-	EBIT growth	5.2%	80.2%	14.4%	-
Inventories	-	-	-	-	EPS growth - recurring	-	(10.6%)	4.1%	-
Other current assets	0	0	0	-	Gross margin	-	-	-	-
Current assets	238	207	170	-	EBIT margin	(123.6%)	(177.4%)	(203.0%)	-
PP&E	0	0	0	-	EBITDA margin	(123.6%)	(177.4%)	(203.0%)	-
Total assets	238	232	233	-	Tax rate	0.0%	0.0%	0.0%	-
Total debt	0	0	0	-	Net margin	(125.5%)	(176.2%)	(195.2%)	-
Total liabilities	77	62	54	-	Net Debt / EBITDA	953.6%	459.7%	331.4%	-
Shareholders' equity	161	170	178	-	Net Debt / Capital (book)	307.8%	561.2%	(2128.9%)	-
Net income (including charges)	(25)	(45)	(49)	-	Return on assets (ROA)	(16.5%)	(19.0%)	(21.3%)	-
D&A	6	8	8	-	Return on equity (ROE)	(22.5%)	(27.0%)	(28.4%)	-
Change in working capital	69	0	0	-	Enterprise value / sales	11.5	10.4	11.8	-
Other	7	0	0	-	Enterprise value / EBITDA	NM	NM	NM	-
Cash flow from operations	63	(31)	(36)	-	Free cash flow yield	25.8%	(6.5%)	(7.1%)	-
Capex	0	0	0	-					
Free cash flow	63	(31)	(36)	-					
Cash flow from investing activities	0	0	0	-					
Cash flow from financing activities	108	0	0	-					
Dividends	-	-	-	-					
Dividend yield	-	-	-	-					

Source: Company reports and J.P. Morgan estimates.

Note: \$ in millions (except per-share data). Fiscal year ends Dec

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bluebird bio (BLUE, BLUE US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	30.65	44.00

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Jul 15, 2013.

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IB clients*	78%	67%	60%

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