

bluebird bio

4Q Snapshot & Model Update

We are updating our BLUE model based on the company's 4Q results (there was no conference call). BLUE continues to make solid progress with its pipeline and we are maintaining our OW rating. 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 appears to be more than just a "big idea" as 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 probability of success.

- **Upcoming events.** We anticipate BLUE announcing the first patient transplanted in its beta thalassemia Northstar Phase 1/2 US trial (HGB-204) with LentiGlobin in early 2014 (the trial has been initiated but we are awaiting the first patient transplant). BLUE expects to provide an early read of some Phase 1/2 data for LentiGlobin in beta-thalassemia (from the HGB-205 and Northstar studies) in late 2014. BLUE also expects to file an IND in the US for sickle cell disease (SCD) in mid-2014, and transplant the first SCD patient in 2H14.
- **4Q results.** 4Q EPS was \$(0.33), slightly wider vs. cons of \$(0.31) and JPMe of \$(0.29). 4Q revs were \$6.3M vs. cons of \$6.4M and JPMe of \$6.3M. 4Q OpEx of \$14.5M was just slightly higher vs. JPMe of \$13.6M. That said, BLUE is not yet an earnings story.
- **Minor estimate revisions; maintaining \$44 YE14 PT.** We have updated our model for 4Q results. Changes were modest and our YE14 PT remains at \$44.
- **Balance sheet update.** BLUE ended 4Q with \$206M in cash (and no debt). Given this solid cash position, BLUE should have sufficient capital to fund operations for at least several years.

Overweight

BLUE, BLUE US

Price: \$25.91

Price Target: \$44.00

Biotechnology

Cory Kasimov ^{AC}

(1-212) 622-5266

cory.w.kasimov@jpmorgan.com

Bloomberg JPMA KASIMOV <GO>

Matthew J. Lowe, Ph.D.

(1-212) 622-0848

matthew.j.lowe@jpmorgan.com

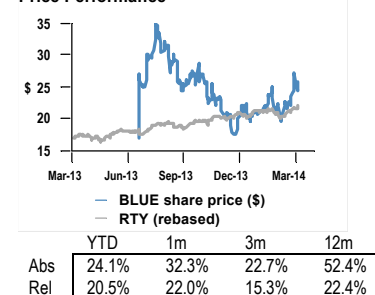
Whitney G Ijem

(1-212) 622-4668

whitney.g.ijem@jpmorgan.com

J.P. Morgan Securities LLC

Price Performance



bluebird bio, Inc. (BLUE;BLUE US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)
EPS reported (\$)			
Q1 (Mar)	-	-	-
Q2 (Jun)	(2.13)	-	-
Q3 (Sep)	(0.26)	-	-
Q4 (Dec)	(0.33)	-	-
FY	(2.02)	(1.20)	(1.58)
Bloomberg EPS FY (\$)	-1.46	-	-1.26

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

Company Data

Price (\$)	25.91
Date Of Price	05 Mar 14
52-week Range (\$)	36.25-17.00
Market Cap (\$ mn)	627.02
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

Program	Event		Expected Timing	Significance
LentiGlobin	1st patient transplanted in Phase 1/2 US study (Northstar) in beta-thalassemia		Early 2014	Low
	File IND for SCD		Mid-2014	Low
	Transplant first SCD patient in HGB-205 Study	✓	2014	Low
	Preliminary 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 Ph1 trial	✓	2016	Low-Medium

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

Upcoming Events

Figure 2: BLUE Upcoming Events

Program	Event		Expected Timing	Significance
LentiGlobin	1st patient transplanted in Phase 1/2 US study (HGB-204) in beta-thalassemia		Early 2014	Low
	File IND for SCD		Mid-2014	Low
	Transplant first SCD patient	✓	2014	Low
	Preliminary Phase 1/2 data for beta-thalassemia from HGB-205 and Northstar trials		Late 2014	High
CAR T-cells	Initiate Ph1 trial	✓	2016	Low-Medium

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

Figure 3: BLUE Income Statement

BlueBird Bio Income Statement																
Cory Kasimov cory.w.kasimov@jpmorgan.com 212.622.5266																
Fiscal Year Ends Dec 31	2012A	1Q13A	2Q13A	3Q13A	4Q13A	2013E	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E	
Total Revenue	\$ 0.3	\$ 1.1	\$ 6.3	\$ 6.4	\$ 6.3	\$ 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	1.1	6.3	6.4	6.3	20.2	6.3	6.3	6.3	6.3	25.3	25.3	5.6	0.3	19.5	
R&D	17.2	5.3	7.2	8.7	9.8	31.0	10.5	11.3	11.3	12.0	45.1	46.0	51.5	57.5	57.5	
Sales and Marketing	-	-	-	-	-	-	-	-	-	-	-	-	21.0	26.3	26.3	
General and Administrative	6.8	2.3	3.3	3.8	4.7	14.1	4.9	5.0	5.2	5.4	20.5	22.6	23.7	24.9	26.1	
Total Operating Expenses	\$ 24.1	\$ 7.6	\$ 10.5	\$ 12.5	\$ 14.5	\$ 45.1	\$ 15.4	\$ 16.3	\$ 16.5	\$ 17.4	\$ 65.6	\$ 68.6	\$ 96.2	\$ 108.6	\$ 116.3	
Operating income	(23.7)	(6.5)	(4.2)	(6.2)	(8.1)	(24.9)	(9.1)	(9.9)	(10.2)	(11.1)	(40.2)	(43.2)	(90.6)	(108.3)	(90.4)	
Other income, net	0.0	(0.06)	(0.39)	0.04	0.0	(0.4)	0.1	0.1	0.1	0.1	0.4	2.2	3.3	3.1	1.1	
Pretax income	(23.7)	(6.5)	(4.6)	(6.1)	(8.1)	(25.3)	(9.0)	(9.8)	(10.1)	(11.0)	(39.8)	(41.0)	(87.3)	(105.2)	(89.3)	
Income Tax (benefit)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	
Net income	\$ (23.7)	\$ (6.5)	\$ (4.6)	\$ (6.1)	\$ (8.1)	\$ (25.3)	\$ (9.0)	\$ (9.8)	\$ (10.1)	\$ (11.0)	\$ (39.8)	\$ (41.0)	\$ (87.3)	\$ (105.2)	\$ (89.3)	
Net Loss applicable to common stockholders	(3.614)	(6.5)	(4.6)	(6.1)	(8.1)	(25.3)	(9.0)	(9.8)	(10.1)	(11.0)	(39.8)	(41.0)	(87.3)	(105.2)	(89.3)	
Average shares Outstanding			2.2	23.6	24.2	12.6	24.6	25.0	25.4	25.8	25.2	26.8	32.4	34.4	36.4	
EPS, Basic and Diluted			(2.13)	(0.26)	(0.33)	(2.02)	(0.36)	(0.39)	(0.40)	(0.43)	(1.58)	(1.53)	(2.69)	(3.06)	(2.45)	
Margin Analysis:																
Gross margin	NM	NM	NM	NM	NM	100%	NM	NM	NM	NM	100%	100%	100%	NM	NM	
Operating margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	
Net margin	NM	NM	NM	NM	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	NM	NM	NM	NM	25%	
R&D as % of tot. revenue	5061.76%	468.86%	114.41%	136.35%	154.14%	153.62%	165.75%	177.58%	178.37%	189.42%	177.78%	181.53%	921.29%	16911.76%	221.68%	
S&M as % of tot. revenue	0.00%	0.00%	0.00%	0.00%	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	NM	NM	NM	NM	
Year-over-year growth:																
Total revenue		1225.88%	NM	NM	NM	5835.59%	462.11%	0.02%	-0.78%	0.00%	25.56%	0.00%	-77.94%	-93.92%	7529.08%	
R&D Expense		36.96%	116.07%	NM	NM	80.14%	98.71%	55.24%	29.80%	22.89%	45.31%	2.11%	11.96%	11.65%	0.00%	
S&M Expense		70.51%	NM	NM	NM	106.34%	110.84%	52.39%	35.56%	15.26%	NM	NM	NM	25.00%	0.00%	
G&A Expense											45.12%	10.00%	5.00%	5.00%	5.00%	
Total operating expenses		45.72%	213.89%	NM	NM	87.60%	102.42%	54.35%	31.56%	20.42%	45.25%	4.58%	40.30%	12.93%	7.12%	
Operating income		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	
Net income		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	
EPS		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	
Tax Rate		NM	NM	NM	NM	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	\$ 42.75	33%	14.2
Risk adjusted NPV analysis	\$ 34.08	33%	11.4
DCF analysis	\$ 53.76	33%	17.9
Total			\$ 43.53
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	1Q14E	2Q14E	3Q14E	4Q14E
Revenues	20	25	25	-	Revenues	-	-	-	-
Cost of products sold	0	0	0	-	Cost of products sold	-	-	-	-
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(14)	(21)	(23)	-	SG&A	-	-	-	-
R&D	(31)	(45)	(46)	-	R&D	-	-	-	-
Operating income	(25)	(40)	(43)	-	Operating income	-	-	-	-
EBITDA	(25)	(40)	(43)	-	EBITDA	-	-	-	-
Net interest (income) / expense	0	0	2	-	Net interest (income) / expense	-	-	-	-
Other income / (expense)	(0)	0	0	-	Other income / (expense)	-	-	-	-
Income taxes	0	0	0	-	Income taxes	-	-	-	-
Net income - GAAP	(25)	(40)	(41)	-	Net income - GAAP	-	-	-	-
Net income - recurring	(25)	(40)	(41)	-	Net income - recurring	-	-	-	-
Diluted shares outstanding	13	25	27	-	Diluted shares outstanding	-	-	-	-
EPS - excluding non-recurring	(2.02)	(1.58)	(1.53)	-	EPS - excluding non-recurring	-	-	-	-
EPS - recurring	(2.02)	(1.58)	(1.53)	-	EPS - recurring	-	-	-	-
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	238	211	184	-	Sales growth	5835.6%	25.6%	0.0%	-
Accounts receivable	0	0	0	-	EBIT growth	5.2%	61.2%	7.5%	-
Inventories	-	-	-	-	EPS growth - recurring	-	(21.6%)	(3.1%)	-
Other current assets	0	0	0	-	Gross margin	-	-	-	-
Current assets	238	211	184	-	EBIT margin	(123.6%)	(158.7%)	(170.5%)	-
PP&E	0	0	0	-	EBITDA margin	(123.6%)	(158.7%)	(170.5%)	-
Total assets	238	237	246	-	Tax rate	0.0%	0.0%	0.0%	-
Total debt	0	0	0	-	Net margin	(125.5%)	(157.2%)	(162.0%)	-
Total liabilities	77	62	54	-	Net Debt / EBITDA	953.6%	526.0%	425.1%	-
Shareholders' equity	161	175	192	-	Net Debt / Capital (book)	307.8%	574.3%	(2294.0%)	-
Net income (including charges)	(25)	(40)	(41)	-	Return on assets (ROA)	(16.5%)	(16.8%)	(17.0%)	-
D&A	6	8	8	-	Return on equity (ROE)	(22.5%)	(23.8%)	(22.4%)	-
Change in working capital	69	0	0	-	Enterprise value / sales	-	-	-	-
Other	7	0	0	-	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	63	(26)	(28)	-	Free cash flow yield	19.3%	(4.0%)	(4.0%)	-
Capex	0	0	0	-					
Free cash flow	63	(26)	(28)	-					
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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