

## bluebird bio

### 3Q Snapshot & Model Update

We are updating our BLUE model based on the company's 3Q results (there was no conference call). BLUE continues to make solid progress with its pipeline (no changes to timelines) 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 both its beta thalassemia HGB-204 Phase 1/2 US trial and beta thalassemia/sickle cell disease HGB-205 Phase 1/2 France trial with LentiGlobin ~YE13/early 2014 (both trials have been initiated and are awaiting the first patient transplant). While 2H13 has been more about trial initiations and getting clinical sites up and running, we could get an early read of some Phase 1/2 data for LentiGlobin in beta-thalassemia in 2H14. BLUE also expects to file an IND in the US for sickle cell disease (SCD) in 2014. In addition, there could also be various clinical data publications over the next 12-18 months that focus additional attention on BLUE's products and gene therapy technology.
- **3Q results.** 3Q EPS was \$(0.26) vs. cons of (\$0.23) and JPMe of (\$0.24). 3Q revs were \$6.4M vs. cons of \$5.3M and JPMe of \$6.3M. 3Q OpEx of \$12.5M was also ~in line with JPMe of \$12.1M. That said, BLUE is not yet an earnings story.
- **Minor estimate revisions; maintaining \$44 PT.** We have updated our model for 3Q results. Changes were modest and our YE14 PT remains at \$44.
- **Balance sheet: solid.** BLUE ended 3Q with \$217M in cash (and no debt). Given this stable cash position, BLUE should have sufficient capital to fund operations for at least several years.

## Overweight

BLUE, BLUE US

Price: \$20.59

Price Target: \$44.00

### Biotechnology

Cory Kasimov <sup>AC</sup>

(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

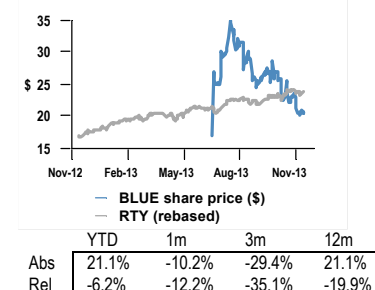
Whitney G Ijem

(1-212) 622-4668

whitney.g.ijem@jpmorgan.com

J.P. Morgan Securities LLC

### Price Performance



### bluebird bio, Inc. (BLUE;BLUE US)

FYE Dec	2013E (Prev)	2013E (Curr)	2014E (Prev)	2014E (Curr)
EPS reported (\$)				
Q1 (Mar)	-	-	-	-
Q2 (Jun)	(2.13)A	(2.13)A	-	-
Q3 (Sep)	(0.24)	(0.26)A	-	-
Q4 (Dec)	(0.30)	(0.29)	-	-
FY	(1.45)	(1.46)	(1.21)	(1.20)
Bloomberg EPS FY (\$)	-	-1.56	-	-1.04

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

### Company Data

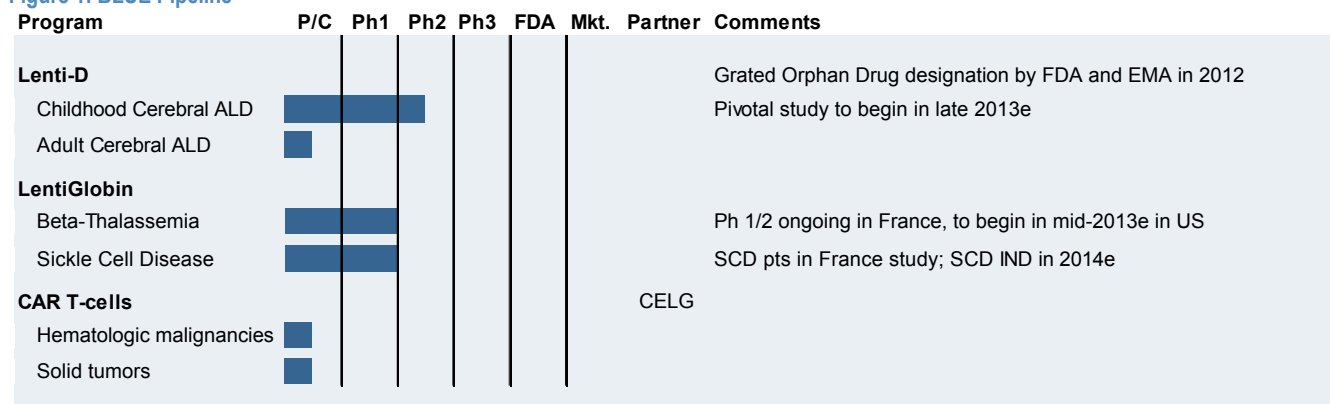
Price (\$)	20.59
Date Of Price	13 Nov 13
52-week Range (\$)	36.25-17.00
Market Cap (\$ mn)	486.40
Fiscal Year End	Dec
Shares O/S (mn)	24
Price Target (\$)	44.00
Price Target End Date	31-Dec-14

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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	1st pt transplanted in Phase 1/2 French study (HGB-205) in beta-thalassemia/SCD	~YE13/1Q14	Medium
	1st pt transplanted in Phase 1/2 US study (HGB-204) in beta-thalassemia	~YE13/1Q14	Medium
	File IND for SCD	2014	Medium
	Preliminary Phase 1/2 data for beta-thalassemia	2H14	High
CAR T-cells	Initiate Ph1 trial	2016	Low-Medium

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

## 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%		
<b>Main value driver</b>	<b>Prob of approval</b>	<b>Peak sales est (avg. scenario)</b>	<b>Avg peak yr</b>
CCALD	50%	\$ 255	2022
B-Thalassemia	25%	\$ 1,175	2023
Sickle Cell Disease	0%	\$ -	-
<b>Valuation methodology</b>	<b>Value</b>	<b>Weighting</b>	<b>Adj. value/ share</b>
P/E 2015	\$ -	0%	\$ -
Real options scenario analysis	\$ 43.84	33%	14.6
Risk adjusted NPV analysis	\$ 34.50	33%	11.5
DCF analysis	\$ 54.84	33%	18.3
Total			\$ 44.39
Catalyst/liquidity discount			0%
<b>YE14 Valuation</b>			<b>\$ 44</b>

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.

Figure 3: BLUE Income Statement

BlueBird Bio Income Statement

Cory Kasimov  
cory.w.kasimov@jpmorgan.com  
212.622.5266

Fiscal Year Ends Dec 31	2011A	2012A	1Q13A	2Q13A	3Q13A	4Q13E	2013E	2014E	2015E	2016E	2017E
Lenti-D - CCALD (\$M)	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -
LentiGlobin - B-Thal (\$M)	-	-	-	-	-	-	-	-	-	-	-
Collaboration revenue (\$M)	-	-	1.0	6.2	6.3	6.3	19.8	25.0	25.0	5.3	-
Research and Licensing Fees (\$M)	0.6	0.3	0.1	0.1	0.1	-	0.3	-	-	-	-
Grant Revenue (\$M)	0.2	-	-	-	-	-	-	-	-	-	-
<b>Total Revenue</b>	<b>\$ 0.9</b>	<b>\$ 0.3</b>	<b>\$ 1.1</b>	<b>\$ 6.3</b>	<b>\$ 6.4</b>	<b>\$ 6.3</b>	<b>\$ 20.1</b>	<b>\$ 25.0</b>	<b>\$ 25.0</b>	<b>\$ 5.3</b>	<b>\$ -</b>
COGS	-	-	-	-	-	-	-	-	-	-	-
<b>Gross Profit</b>	<b>11.4</b>	<b>17.2</b>	<b>5.3</b>	<b>7.2</b>	<b>8.7</b>	<b>10.1</b>	<b>31.3</b>	<b>42.0</b>	<b>46.0</b>	<b>51.5</b>	<b>57.5</b>
R&D	-	-	-	-	-	-	-	-	-	-	-
Sales and Marketing	-	-	-	-	-	-	-	-	-	-	-
General and Administrative	4.6	6.8	2.3	3.3	3.8	3.5	12.9	14.2	15.7	16.4	17.3
<b>Total Operating Expenses</b>	<b>\$ 16.0</b>	<b>\$ 24.1</b>	<b>\$ 7.6</b>	<b>\$ 10.5</b>	<b>\$ 12.5</b>	<b>\$ 13.6</b>	<b>\$ 44.3</b>	<b>\$ 56.2</b>	<b>\$ 61.7</b>	<b>\$ 88.9</b>	<b>\$ 101.0</b>
Operating income	(15.1)	(23.7)	(6.5)	(4.2)	(6.2)	(7.4)	(24.2)	(31.2)	(36.7)	(83.7)	(101.0)
Other income, net	(0.5)	0.0	(0.06)	(0.39)	0.04	0.4	(0.0)	1.9	2.4	3.5	3.1
Pretax income	(15.6)	(23.7)	(6.5)	(4.6)	(6.1)	(6.9)	(24.2)	(29.3)	(34.3)	(80.2)	(97.9)
Income Tax (benefit)	-	-	-	-	-	-	-	-	-	-	-
<b>Net Income</b>	<b>\$ (15.6)</b>	<b>\$ (23.7)</b>	<b>\$ (6.5)</b>	<b>\$ (4.6)</b>	<b>\$ (6.1)</b>	<b>\$ (6.9)</b>	<b>\$ (24.2)</b>	<b>\$ (29.3)</b>	<b>\$ (34.3)</b>	<b>\$ (80.2)</b>	<b>\$ (97.9)</b>
<b>Net Loss applicable to common stockholders</b>	<b>(20.6)</b>	<b>(3.614)</b>	<b>\$ (6.5)</b>	<b>\$ (4.6)</b>	<b>\$ (6.1)</b>	<b>\$ (6.9)</b>	<b>(24.2)</b>	<b>(29.3)</b>	<b>(34.3)</b>	<b>(80.2)</b>	<b>(97.9)</b>
Average shares Outstanding				2.2	23.6	23.9	16.5	24.4	24.9	29.2	29.7
<b>EPS, Basic and Diluted</b>				<b>(2.13)</b>	<b>(0.26)</b>	<b>(0.29)</b>	<b>(1.46)</b>	<b>(1.20)</b>	<b>(1.38)</b>	<b>(2.75)</b>	<b>(3.29)</b>
<b>Margin Analysis:</b>											
Gross margin		NM	NM	NM	NM	NM	100%	100%	100%	100%	NM
Operating margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
<b>Cost Analysis:</b>											
COGS as % of tot. prod. sales		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
R&D as % of tot. revenue		5061.76%	468.86%	114.41%	136.35%	161.60%	155.94%	168.00%	184.00%	980.95%	NM
S&M as % of tot. revenue		0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%	400.00%	NM
G&A as % of tot. revenue		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
<b>Year-over-year growth:</b>											
Total revenue			1225.88%	NM	NM	NM	5810.59%	24.40%	0.00%	-79.00%	NM
R&D Expense			36.96%	116.07%	NM	NM	82.09%	34.03%	9.52%	11.96%	11.65%
S&M Expense			70.51%	NM	NM	NM	NM	NM	NM	NM	25.00%
G&A Expense							89.03%	10.00%	10.00%	5.00%	5.00%
Total operating expenses			45.72%	213.89%	NM	NM	84.06%	27.00%	9.64%	44.25%	13.57%
Operating income			NM	NM	NM	NM	NM	NM	NM	NM	NM
Net income			NM	NM	NM	NM	NM	NM	NM	NM	NM
EPS			NM	NM	NM	NM	NM	NM	NM	NM	NM
<b>Tax Rate</b>			NM	NM	NM	NM	NM	NM	NM	NM	NM

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

## bluebird bio: Summary of Financials

Income Statement - Annual	FY12A	FY13E	FY14E	FY15E	Income Statement - Quarterly	1Q13A	2Q13A	3Q13A	4Q13E
Revenues	0	20	25	25	Revenues	1A	6A	6A	6
Cost of products sold	0	0	0	0	Cost of products sold	0A	0A	0A	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(7)	(13)	(14)	(16)	SG&A	(2)A	(3)A	(4)A	(4)
R&D	(17)	(31)	(42)	(46)	R&D	(5)A	(7)A	(9)A	(10)
Operating income	(24)	(24)	(31)	(37)	Operating income	(6)A	(4)A	(6)A	(7)
EBITDA	(24)	(24)	(31)	(37)	EBITDA	(6)A	(4)A	(6)A	(7)
Net interest (income) / expense	0	0	2	2	Net interest (income) / expense	0A	0A	0A	0
Other income / (expense)	0	(0)	0	0	Other income / (expense)	(0)A	(0)A	0A	0
Income taxes	0	0	0	0	Income taxes	0A	0A	0A	0
Net income - GAAP	(1)	(24)	(29)	(34)	Net income - GAAP	(7)A	(5)A	(6)A	(7)
Net income - recurring	(4)	(24)	(29)	(34)	Net income - recurring	(7)A	(5)A	(6)A	(7)
Diluted shares outstanding	0	17	24	25	Diluted shares outstanding	0A	2A	24A	24
EPS - excluding non-recurring	-	(1.46)	(1.20)	(1.38)	EPS - excluding non-recurring	-	(2.13)A	(0.26)A	(0.29)
EPS - recurring	-	(1.46)	(1.20)	(1.38)	EPS - recurring	-	(2.13)A	(0.26)A	(0.29)
Balance Sheet and Cash Flow Data	FY12A	FY13E	FY14E	FY15E	Ratio Analysis	FY12A	FY13E	FY14E	FY15E
Cash and cash equivalents	67	239	223	202	Sales growth	(61.5%)	5810.6%	24.4%	0.0%
Accounts receivable	0	0	0	0	EBIT growth	56.6%	2.0%	29.2%	17.4%
Inventories	-	-	-	-	EPS growth - recurring	-	-	(17.8%)	14.6%
Other current assets	1	0	0	0	Gross margin	-	-	-	-
Current assets	68	239	223	202	EBIT margin	(6975.3%)	(120.3%)	(124.9%)	(146.6%)
PP&E	1	0	0	0	EBITDA margin	(6975.3%)	(120.3%)	(124.9%)	(146.6%)
Total assets	69	239	248	265	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	0	0	0	0	Net margin	(1062.8%)	(120.4%)	(117.2%)	(137.0%)
Total liabilities	5	77	62	54	Net Debt / EBITDA	282.6%	988.4%	714.5%	551.5%
Shareholders' equity	64	162	186	210	Net Debt / Capital (book)	2296.5%	309.3%	605.9%	(2524.5%)
Net income (including charges)	(24)	(24)	(29)	(34)	Return on assets (ROA)	(7.2%)	(15.7%)	(12.0%)	(13.4%)
D&A	0	6	8	8	Return on equity (ROE)	(8.0%)	(21.4%)	(16.8%)	(17.3%)
Change in working capital	2	69	0	0	Enterprise value / sales	-	-	-	-
Other	1	7	0	0	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	(21)	64	(16)	(21)	Free cash flow yield	-	18.8%	(3.2%)	(4.1%)
Capex	(1)	0	0	0					
Free cash flow	(22)	64	(16)	(21)					
Cash flow from investing activities	3	0	0	0					
Cash flow from financing activities	60	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

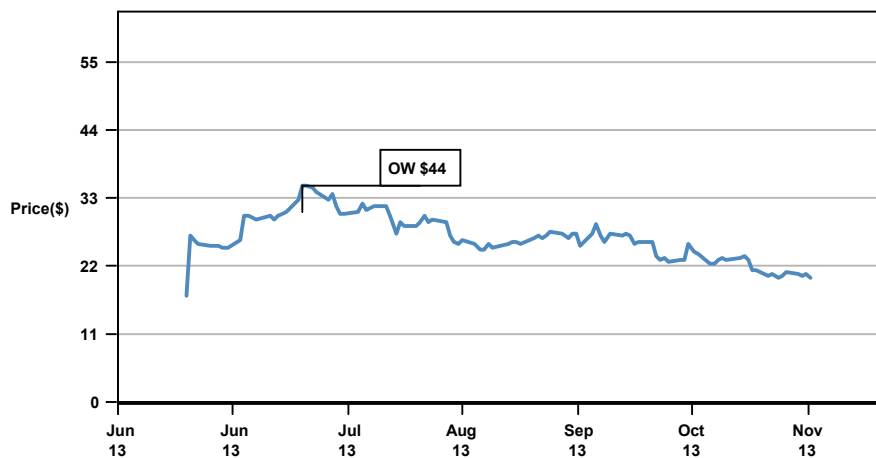
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bluebird bio (BLUE, BLUE US) Price Chart



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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