

## bluebird bio, Inc. (BLUE)

Overweight

REVISED: Raising PT Already As LentiGlobin Data Pivots The Platform

### CONCLUSION

While we just initiated coverage of BLUE last Friday, we already have reason to take our price target up substantially to reflect the stronger-than-expected beta-thalassemia (BT) data reported over the weekend. These results not only establish proof of concept in BT, but also increase the probability that success will be seen in sickle cell disease (SCD). Our PT is now \$51 vs prior \$34, as we increase our assumed penetration into the BT market (we now model 128 BT patients treated in 2022).

*Note: the second bullet was revised to clarify that the same product (LentiGlobin) will be used for the BT and SCD indications.*

- **Impressive, validating data:** The BT data released at EHA represent a great improvement to the first study (LG001), which used a precursor to LentiGlobin. The two patients in the HGB-205 study experienced rapid production of therapeutic hemoglobin (Hb) within weeks, as opposed to one year, which was seen in the LG001 study. Both patients now maintain near-normal Hb levels without transfusion support. One patient is producing an impressive 6.6g/dL of the therapeutic Hb gene at 4.5 months after transplant, and the other is producing 4.2 g/dL of the therapeutic Hb gene at 2 months after transplant. The next clinical update can be expected by YE14, which could reveal data from more patients over a longer follow-up period.
- **If it works here, it should work in sickle:** The new vector in the HGB-205 study employs an improved manufacturing process to produce a more potent and pure drug, with greater transduction efficiency. Optimizing the vector construct is an iterative process and can have a great impact on the therapeutic effect. Because the same product (LentiGlobin) will be used for treating SCD patients (only the transgene changes), we can extrapolate that these patients should experience a therapeutic effect similar to that seen in the BT patients. LentiGlobin was well tolerated by the BT patients, with no adverse events reported.
- **Good for the field:** Successes with any gene therapy program should help drive investor interest in other gene therapy 'pure-plays,' and hopefully interest in our June 23rd GenomeRx Symposium. BLUE is available for 1x1 meetings with investors on the following day (June 24th).
- **Changes to our model and valuation:** We are increasing penetration assumptions into BT but still do not include SCD in our model. We are also increasing OPEX assumptions to reflect greater commercial needs and investments in R&D. Our growth rate remains 8% since we still see considerable opportunity for the platform, including SCD and beyond. Our 2022 tax rate falls from 25% to 20% because BT has greater prevalence ex-U.S., which should help lower net taxes. Our discount rate remains 11%.

### RISKS TO ACHIEVEMENT OF PRICE TARGET

BLUE or the gene therapy field may face development or regulatory setbacks.

### COMPANY DESCRIPTION

BLUE is one of the pioneers in gene therapy.

YEAR	REVENUE ( m)						EARNINGS PER SHARE ( )					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2013A	1.1	6.3	6.4	6.4	20.2	31.6x	(19.94)	(2.13)	(0.26)	(0.34)	(2.02)	NM
2014E	6.3A	6.0	6.0	6.0	24.3	26.3x	(0.44)A	(0.44)	(0.43)	(0.43)	(1.74)	NM
2015E	—	—	—	—	21.0	30.4x	—	—	—	—	(1.84)	NM

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bluebird bio, Inc.

PRICE: US\$26.09

TARGET: US\$51.00

DCF through 2022 using 11% disc rate, 8% terminal growth & 36x terminal multiple.

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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	US\$34.00	US\$51.00
FY14E Rev (mil)	—	24.3
FY15E Rev (mil)	—	21.0
FY14E EPS	—	(1.74)
FY15E EPS	—	(1.84)
52-Week High / Low	US\$36.25 / US\$17.00	
Shares Out (mil)	24.5	
Market Cap. (mil)	US\$639.2	
Avg Daily Vol (ooo)	233	
Net Cash Per Share	US\$7.80	
Yield	0.00%	
Fiscal Year End	Dec	

### Price Performance - 1 Year



Source: Bloomberg

Program	Indication	Type	Event	Expected Timing
Lenti-D	CCALD	Clinical	Initiate enrollment in EU sites	2014
		Clinical	Complete Enrollment in Starbeam Study	2015
		Regulatory	File BLA	2016/2017
LentiGlobin	Beta-Thalassemia	Clinical	Additional data from Northstar and HGB-205	YE14
	Sickle Cell Disease	Clinical	Initiate enrollment in HGB-206	2H14
		Clinical	Prelim. Data from HGB-205 and HGB-206	2015

Source: PJC and Company reports

Discounted Cash Flow (DCF) Analysis	
<i>Assumed Discount Rate (%)</i>	<i>11.0%</i>
Discounted Net Cash Flow (2014-'22)	-\$74
<i>Terminal Growth Rate (%)</i>	<i>8%</i>
<b>Implied Terminal Year FCF Multiple</b>	<b>36.0x</b>
NPV of FCF	\$1,652
<i>Terminal value as % of total</i>	<i>104.5%</i>
Net Cash	159
Shares Outstanding 2018E (million)	35.7
<b>Price Target</b>	<b>\$51</b>
Current Price	\$35.42
<i>Mkt Cap, Current Share Count</i>	<i>\$855</i>
<i>Implied Mkt Cap, Price Target</i>	<i>\$1,224</i>
<b>Implied Multiple on 2020 Rev</b>	<b>3.4x</b>

Source: Company Reports and Piper Jaffray.

BLUEPrice Target Sensitivity Analysis					
		Discount Rate			
		10.0%	11.0%	12.0%	13.0%
Terminal Growth	5%	\$33	\$25	\$20	\$16
	6%	\$41	\$30	\$23	\$19
	7%	\$55	\$38	\$28	\$22
	8%	\$83	<b>\$51</b>	\$35	\$26
	9%	\$165	\$76	\$47	\$32
<b>Growth rate in 2022:</b>		<b>94.3%</b>			

Source: Company Reports and Piper Jaffray.

BLUEPotential Upside From Current Levels					
		Discount Rate			
		9.0%	10.0%	11.0%	12.0%
Terminal Growth	5.0%	(7%)	(29%)	(43%)	(54%)
	6.0%	16%	(14%)	(34%)	(48%)
	7.0%	55%	7%	(21%)	(39%)
	8.0%	133%	<b>43%</b>	(1%)	(27%)
	9.0%	367%	115%	32%	(8%)

Source: Company Reports and Piper Jaffray.

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BLUE Quarterly P&L	2012A	1Q13A	2Q13A	3Q13A	4Q13A	2013A	1Q14A	2Q14E	3Q14E	4Q14E	2014E
Collaboration revenue	0.1	1.0	6.2	6.3	6.3	19.8	6.3	6.0	6.0	6.0	24.3
Research and license fees	0.7	0.1	0.1	0.1	0.1	0.4	0.1	0.0	0.0	0.0	0.1
Product revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Other product-associated revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
<b>Total Revenue</b>	<b>0.7</b>	<b>1.1</b>	<b>6.3</b>	<b>6.4</b>	<b>6.4</b>	<b>20.2</b>	<b>6.3</b>	<b>6.0</b>	<b>6.0</b>	<b>6.0</b>	<b>24.3</b>
<b>Operating Expenses:</b>											
COGS	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
<i>% product sales</i>	<i>7%</i>	<i>5%</i>	<i>5%</i>	<i>5%</i>	<i>5%</i>	<i>7%</i>	<i>5%</i>	<i>5%</i>	<i>5%</i>	<i>5%</i>	<i>7%</i>
R&D	17.2	5.3	7.2	8.7	9.8	31.0	11.5	12.0	12.0	12.0	47.5
<i>% Revenue</i>	<i>2326%</i>	<i>469%</i>	<i>114%</i>	<i>136%</i>	<i>152%</i>	<i>153%</i>	<i>181%</i>	<i>200%</i>	<i>200%</i>	<i>200%</i>	<i>195%</i>
General and administrative	6.8	2.3	3.3	3.8	4.7	14.1	5.5	5.5	5.5	5.5	22.0
<i>% Revenue</i>	<i>925%</i>	<i>206%</i>	<i>52%</i>	<i>60%</i>	<i>73%</i>	<i>70%</i>	<i>87%</i>	<i>92%</i>	<i>92%</i>	<i>92%</i>	<i>91%</i>
<b>Total Operating Expenses</b>	<b>24.1</b>	<b>7.6</b>	<b>10.5</b>	<b>12.5</b>	<b>14.5</b>	<b>45.1</b>	<b>17.0</b>	<b>17.5</b>	<b>17.5</b>	<b>17.5</b>	<b>69.5</b>
<b>Operating Income (GAAP)</b>	<b>(23.3)</b>	<b>(6.5)</b>	<b>(4.2)</b>	<b>(6.2)</b>	<b>(8.0)</b>	<b>(24.9)</b>	<b>(10.7)</b>	<b>(11.5)</b>	<b>(11.5)</b>	<b>(11.5)</b>	<b>(45.2)</b>
Interest income	0.1	0.0	0.0	0.0	0.0	0.0	0.0	0.6	0.7	0.8	2.1
Interest expense/other	0.0	(0.1)	(0.4)	0.0	0.0	(0.4)	0.1	0.1	0.1	0.1	0.4
<b>Pretax Income (loss)</b>	<b>(23.7)</b>	<b>(6.5)</b>	<b>(4.6)</b>	<b>(6.1)</b>	<b>(8.0)</b>	<b>(25.3)</b>	<b>(10.6)</b>	<b>(10.8)</b>	<b>(10.7)</b>	<b>(10.6)</b>	<b>(42.7)</b>
Net Income as Reported/GAAP	(3.6)	(6.5)	(4.6)	(6.1)	(8.0)	(25.3)	(10.6)	(10.8)	(10.7)	(10.6)	(42.7)
<b>Diluted EPS (as reported/GAAP)</b>	<b>(\$13.79)</b>	<b>(\$19.94)</b>	<b>(\$2.13)</b>	<b>(\$0.26)</b>	<b>(\$0.34)</b>	<b>(\$2.02)</b>	<b>(\$0.44)</b>	<b>(\$0.44)</b>	<b>(\$0.43)</b>	<b>(\$0.43)</b>	<b>(\$1.74)</b>
Weighted average shares outstanding	0.3	0.3	2.2	23.6	23.8	12.6	24.1	24.4	24.6	24.9	24.5

Source: Company reports and PJC Estimates

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Current disclosure information for this company can be found at <http://www.piperjaffray.com/researchdisclosures>.

Proprietary to Piper Jaffray & Co. June 16, 2014

<b>BLUE Revenue Model</b>	<b>2012A</b>	<b>2013A</b>	<b>2014E</b>	<b>2015E</b>	<b>2016E</b>	<b>2017E</b>	<b>2018E</b>	<b>2019E</b>	<b>2020E</b>	<b>2021E</b>	<b>2022E</b>
W/W ALD Incidence	350	350	350	365	385	400	410	420	440	460	470
W/W CCALD Incidence	35%	35%	35%	35%	35%	35%	35%	40%	45%	45%	45%
W/W AMN Incidence	40%	40%	40%	40%	40%	40%	40%	40%	40%	40%	40%
CCALD Patients	123	123	123	128	135	140	144	168	198	207	212
AMN Patients	140	140	140	146	154	160	164	168	176	184	188
<i>Lenti-D Penetration into CCALD</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>5%</i>	<i>20%</i>	<i>45%</i>	<i>55%</i>	<i>60%</i>
CCALD Patients treated with Lenti-D	0	0	0	0	0	0	7	34	89	114	127
<i>Lenti-D Penetration into AMN</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>3%</i>	<i>4%</i>	<i>7%</i>
AMN Patients treated with Lenti-D	0	0	0	0	0	0	0	0	5	7	13
Lenti-D Price	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$1.3	\$1.3	\$1.3	\$1.3	\$1.3
W/W Lenti-D Revenue	\$0	\$0	\$0	\$0	\$0	\$0	\$9	\$44	\$123	\$158	\$182
US/EU Beta-Thalassemia Major Prevalence	15,000	15,000	15,000	15,000	15,000	15,000	15,000	15,000	15,000	15,000	15,000
<i>LentiGlobin Penetration</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0.2%</i>	<i>0.4%</i>	<i>0.9%</i>
Patients treated with LentiGlobin	0	0	0	0	0	0	0	0	30	60	128
LentiGlobin Price	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$1.2	\$1.2	\$1.2
LentiGlobin Revenue	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$36	\$72	\$153
<b>TOTAL REVENUE</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$9.3</b>	<b>\$43.7</b>	<b>\$158.7</b>	<b>\$229.6</b>	<b>\$335.1</b>

Source: Company reports and PJC Estimates

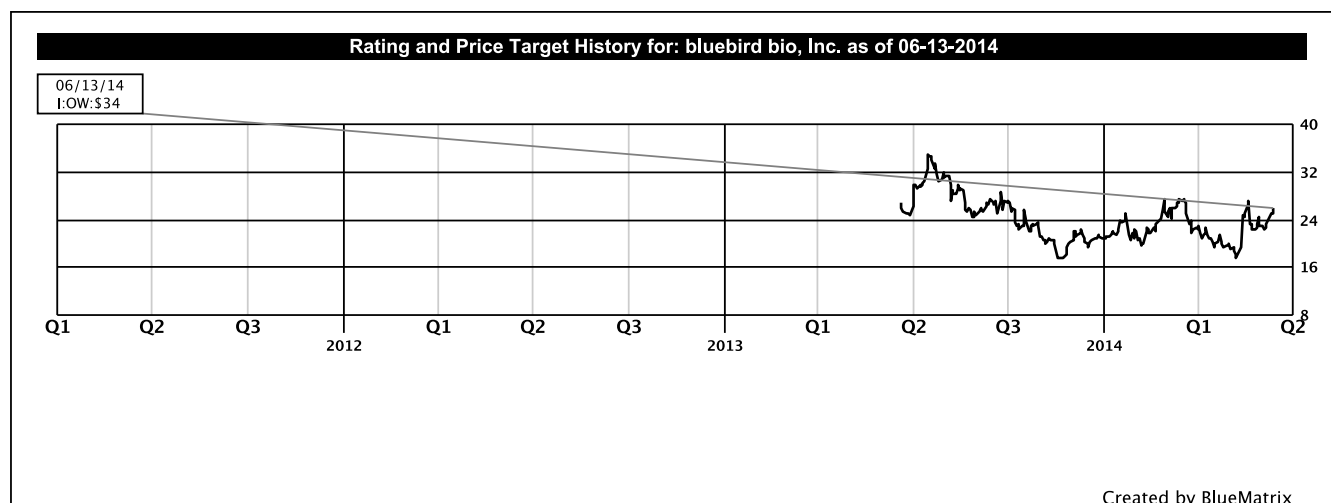
<b>BLUE P&amp;L (MM)</b>	<b>2012A</b>	<b>2013A</b>	<b>2014E</b>	<b>2015E</b>	<b>2016E</b>	<b>2017E</b>	<b>2018E</b>	<b>2019E</b>	<b>2020E</b>	<b>2021E</b>	<b>2022E</b>
Collaboration revenue	0.1	19.8	24.3	20.0	20.0	20.0	23.0	25.0	25.0	25.0	25.0
Research and license fees	0.7	0.4	0.1	1.0	1.0	1.0	1.0	1.0	1.0	1.0	1.0
Product revenue	0.0	0.0	0.0	0.0	0.0	0.0	9.3	43.7	158.7	229.6	335.1
Other product-associated revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
<b>Total Revenue</b>	<b>\$0.7</b>	<b>\$20.2</b>	<b>\$24.3</b>	<b>\$21.0</b>	<b>\$21.0</b>	<b>\$21.0</b>	<b>\$33.3</b>	<b>\$69.7</b>	<b>\$184.7</b>	<b>\$255.6</b>	<b>\$361.1</b>
COGS	0.0	0.0	0.0	0.0	0.0	0.0	0.7	3.1	11.1	16.1	23.5
<i>% product sales</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>	<i>7%</i>
R&D	17.2	31.0	47.5	48.0	48.0	48.0	50.0	50.0	60.0	65.0	75.0
General and administrative	6.8	14.1	22.0	25.0	25.0	35.0	60.0	65.0	85.0	110.0	120.0
<b>Total Operating Expenses</b>	<b>\$24.1</b>	<b>\$45.1</b>	<b>\$69.5</b>	<b>\$73.0</b>	<b>\$73.0</b>	<b>\$83.0</b>	<b>\$110.7</b>	<b>\$118.1</b>	<b>\$156.1</b>	<b>\$191.1</b>	<b>\$218.5</b>
<b>Operating Income (GAAP)</b>	<b>(\$23.3)</b>	<b>(\$24.9)</b>	<b>(\$45.2)</b>	<b>(\$52.0)</b>	<b>(\$52.0)</b>	<b>(\$62.0)</b>	<b>(\$77.3)</b>	<b>(\$48.4)</b>	<b>\$28.6</b>	<b>\$64.5</b>	<b>\$142.6</b>
Interest income	0.1	0.0	2.1	3.0	3.0	4.0	4.0	5.0	5.0	6.0	7.0
Interest expense/other	0.0	(0.4)	0.4	0.5	0.5	0.5	1.0	1.0	1.0	1.0	1.0
<b>Pretax Income (loss)</b>	<b>(23.7)</b>	<b>(25.3)</b>	<b>(42.7)</b>	<b>(48.5)</b>	<b>(48.5)</b>	<b>(57.5)</b>	<b>(72.3)</b>	<b>(42.4)</b>	<b>34.6</b>	<b>71.5</b>	<b>150.6</b>
<i>Tax rate</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>15%</i>	<i>20%</i>
<b>Taxes</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>10.7</b>	<b>30.1</b>
Net Income as Reported/GAAP	(3.6)	(25.3)	(42.7)	(48.5)	(48.5)	(57.5)	(72.3)	(42.4)	34.6	60.8	120.5
<b>Diluted EPS (as reported/GAAP)</b>	<b>(\$13.79)</b>	<b>(\$2.02)</b>	<b>(\$1.74)</b>	<b>(\$1.84)</b>	<b>(\$1.62)</b>	<b>(\$1.83)</b>	<b>(\$2.02)</b>	<b>(\$1.14)</b>	<b>\$0.86</b>	<b>\$1.47</b>	<b>\$2.81</b>
Weighted average shares outstanding	0.3	12.6	24.5	26.3	29.9	31.4	35.7	37.2	40.2	41.4	42.8

Source: Company reports and PJC Estimates

<b>BLUE Cash Flow Statement</b>	<b>2012A</b>	<b>2013A</b>	<b>2014E</b>	<b>2015E</b>	<b>2016E</b>	<b>2017E</b>	<b>2018E</b>	<b>2019E</b>	<b>2020E</b>	<b>2021E</b>	<b>2022E</b>
<b>Net income</b>	<b>(4)</b>	<b>(25)</b>	<b>(43)</b>	<b>(49)</b>	<b>(49)</b>	<b>(58)</b>	<b>(72)</b>	<b>(42)</b>	<b>35</b>	<b>61</b>	<b>120</b>
Depreciation/amortization	0	1	1	1	1	1	2	2	2	2	3
Stock based comp	1	6	7	9	10	13	16	17	17	18	20
Remeasure of warrants	(0)	0									
Loss on disposal	0	0									
Change in NWC	2	61	(8)	(8)	(10)	(15)	(15)	(5)	(5)	(5)	0
<b>Net Cash Provided by Operating Activities</b>	<b>(21)</b>	<b>43.5</b>	<b>(43)</b>	<b>(47)</b>	<b>(48)</b>	<b>(59)</b>	<b>(69)</b>	<b>(28)</b>	<b>49</b>	<b>76</b>	<b>143</b>
Capital expenditures	0	(9)	(4)	(5)	(4)	(4)	(4)	(4)	(4)	(4)	(4)
<b>Free Cash Flow</b>	<b>(21)</b>	<b>35</b>	<b>(47)</b>	<b>(52)</b>	<b>(52)</b>	<b>(63)</b>	<b>(73)</b>	<b>(32)</b>	<b>45</b>	<b>72</b>	<b>139</b>
<b>Net Cash From Financing</b>	<b>60</b>	<b>106</b>	<b>1</b>	<b>2</b>	<b>75</b>	<b>2</b>	<b>100</b>	<b>2</b>	<b>2</b>	<b>2</b>	<b>3</b>
<b>Cash and Cash Equivalents</b>	<b>67</b>	<b>206</b>	<b>159</b>	<b>109</b>	<b>133</b>	<b>72</b>	<b>99</b>	<b>69</b>	<b>115</b>	<b>189</b>	<b>332</b>

Sources: Company Reports and Piper Jaffray

## IMPORTANT RESEARCH DISCLOSURES



Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written during the past three years.

## Legend:

I: Initiating Coverage  
 R: Resuming Coverage  
 T: Transferring Coverage  
 D: Discontinuing Coverage  
 S: Suspending Coverage  
 OW: Overweight  
 N: Neutral  
 UW: Underweight  
 NA: Not Available  
 UR: Under Review

Distribution of Ratings/IB Services Piper Jaffray				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
<b>BUY [OW]</b>	355	61.95	87	24.51
<b>HOLD [N]</b>	203	35.43	21	10.34
<b>SELL [UW]</b>	15	2.62	0	0.00

Note: Distribution of Ratings/IB Services shows the number of companies currently in each rating category from which Piper Jaffray and its affiliates received compensation for investment banking services within the past 12 months. FINRA rules require disclosure of which ratings most closely correspond with "buy," "hold," and "sell" recommendations. Piper Jaffray ratings are not the equivalent of buy, hold or sell, but instead represent recommended relative weightings. Nevertheless, Overweight corresponds most closely with buy, Neutral with hold and Underweight with sell. See Stock Rating definitions below.

**Analyst Certification — Joshua E. Schimmer, MD, Sr Research Analyst**  
**— Kristina N. Cibor, Research Analyst**

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