

bluebird bio, Inc. (BLUE)

Overweight

Drawing The Beta-Thal Lines In The Sand; Raising PT To \$58

CONCLUSION

We believe we have found a natural split in the beta thal market that yields opportunity for BLUE & BIIB should the latter's program prove competitive. Albeit prematurely, investors are trying to pick a 'winner' between these, and this was one of our focuses after our GenomeRx Symposium. Of course we still need to see BIIB clinical data (2015) and more data from BLUE, but we postulate a plausible market split based on theoretical risks of these programs in males vs females. We believe this helps protect BLUE downside. Our PT is now \$58 (prior \$51; discount rate from 11% to 10.5%).

- **The debate:** BLUE bulls argue a 'bird in the hand' as data from 2 beta-thal patients set a very high bar and leukemic risk with lenti is very low. BLUE bears argue the theoretical risk of leukemia with lentivirus integration broadly limits its potential vs the BIIB/(SGMO) gene editing approach (which doesn't involve an integration step).
- **BIIB/SGMO approach:** We won't see data until 2015, but the science is elegant. By knocking out the BCL11a switch from fetal to adult hemoglobin (Hb), this program should increase levels of fetal Hb to replace defective beta-Hb and reduce/eliminate the need for transfusions. Because this is a gene editing approach and not an integration approach, there is not a theoretical leukemia risk. This approach (and LentiGlobin) should also have benefit in sickle cell anemia, a related hemoglobinopathy.
- **Why we switch from fetal to adult hemoglobin (Hb):** It turns out a gradient in hemoglobin oxygen affinity is required for a fetus to extract oxygen in utero. This is accomplished by having fetuses predominant in fetal hemoglobin (higher affinity) and pregnant mothers predominant in adult hemoglobin (lower affinity).
- **The risk of fetal Hb in adults:** Hereditary Persistence of Fetal Hemoglobin (HPFH) is a condition in which adults have persistently high levels of fetal hemoglobin. They tend to be asymptomatic. However, Murji et al (Journal of Maternal-Fetal & Neonatal Medicine 2011) found a 100% rate of intrauterine growth restriction or small for gestational age births among pregnancies in HPFH mothers with HbF >70%, presumably reflecting the lesser oxygen affinity gradient and theoretically, although these babies were otherwise healthy, there could be fetal risk.
- **What it means:** If otherwise comparable (a big 'if'), we believe the theoretical fetal/pregnancy risk is a concern for BIIB which could offset a theoretical risk of leukemia for some females, driving them to opt for LentiGlobin. In contrast a leukemia risk counter-detail may prove more consistently effective for BIIB in males where pregnancy isn't a concern.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Failure to achieve clinical, regulatory and development targets. Insertional mutagenesis.

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	47.9x	(19.94)	(2.13)	(0.26)	(0.34)	(2.02)	NM
2014E	6.3A	6.0	6.0	6.0	24.3	39.8x	(0.44)A	(0.44)	(0.43)	(0.43)	(1.74)	NM
2015E	—	—	—	—	21.0	46.1x	—	—	—	—	(1.84)	NM

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bluebird bio, Inc.

PRICE: US\$39.52

TARGET: US\$58.00

DCF through 2022 using 10.5% disc rate,
8% terminal growth & 36x terminal multiple.

Joshua E. Schimmer, MD

Sr Research Analyst, Piper Jaffray & Co.

212 284-9322, joshua.e.schimmer@pjc.com

Kristina N. Cabor, Research Analyst

617 654-0727, kristina.n.cabor@pjc.com

Changes	Previous	Current
Rating	—	Overweight
Price Tgt	US\$51.00	US\$58.00
FY14E Rev (mil)	—	24.3
FY15E Rev (mil)	—	21.0
FY14E EPS	—	(1.74)
FY15E EPS	—	(1.84)
52-Week High / Low	US\$41.75 / US\$17.03	
Shares Out (mil)	24.5	
Market Cap. (mil)	US\$968.2	
Avg Daily Vol (ooo)	364	
Net Cash Per Share	US\$7.80	
Yield	0.00%	
Fiscal Year End	Dec	

Price Performance - 1 Year



Source: Bloomberg

Increase in Price Target to \$58 from \$51

Our price target remains based on a DCF through 2022 using 8% terminal growth and a 36x terminal multiple. However we have lowered our discount rate to 10.5% from 11% due to our appreciation for a plausible advantage in almost half the BT and sickle cell indications which makes LentiGlobin less vulnerable to a competitive threat from BIIB.

Biogen Idec Rating, Price Target and Risks

Biogen Idec (BIIB, \$314.76) is rated Overweight with a price target of \$370 based on a DCF to 2022 with a 10% discount rate, 5% terminal growth rate. Risks include: failure of Tecfidera and BIIB's overall MS franchise to achieve revenue projections; new safety signals with Tecfidera; pipeline setbacks.

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>10.5%</i>
Discounted Net Cash Flow (2014-'22)	-\$78
<i>Terminal Growth Rate (%)</i>	<i>8%</i>
Implied Terminal Year FCF Multiple	43.2x
NPV of FCF	\$1,909
<i>Terminal value as % of total</i>	<i>104.1%</i>
Net Cash	159
Shares Outstanding 2018E (million)	35.7
Price Target	\$58
Current Price	\$35.42
<i>Mkt Cap, Current Share Count</i>	<i>\$855</i>
<i>Implied Mkt Cap, Price Target</i>	<i>\$1,398</i>
Implied Multiple on 2020 Rev	3.9x

Source: Company Reports and Piper Jaffray.

BLUEPrice Target Sensitivity Analysis					
	Discount Rate				
		10.0%	10.5%	11.0%	11.5%
Terminal Growth	5%	\$30	\$26	\$23	\$20
	6%	\$38	\$32	\$28	\$24
	7%	\$50	\$41	\$35	\$30
	8%	\$75	\$58	\$46	\$38
	9%	\$151	\$97	\$70	\$53
Growth rate in 2022:		77.6%			

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%	(15%)	(26%)	(35%)	(42%)
	6.0%	6%	(9%)	(22%)	(32%)
	7.0%	42%	17%	(2%)	(16%)
	8.0%	113%	63%	31%	8%
	9.0%	327%	173%	96%	51%

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
Total Revenue	0.7	1.1	6.3	6.4	6.4	20.2	6.3	6.0	6.0	6.0	24.3
Operating Expenses:											
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>
Total Operating Expenses	24.1	7.6	10.5	12.5	14.5	45.1	17.0	17.5	17.5	17.5	69.5
Operating Income (GAAP)	(23.3)	(6.5)	(4.2)	(6.2)	(8.0)	(24.9)	(10.7)	(11.5)	(11.5)	(11.5)	(45.2)
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
Pretax Income (loss)	(23.7)	(6.5)	(4.6)	(6.1)	(8.0)	(25.3)	(10.6)	(10.8)	(10.7)	(10.6)	(42.7)
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)
Diluted EPS (as reported/GAAP)	(\$13.79)	(\$19.94)	(\$2.13)	(\$0.26)	(\$0.34)	(\$2.02)	(\$0.44)	(\$0.44)	(\$0.43)	(\$0.43)	(\$1.74)
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

Joshua Schimmer: 212-284-9322

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BLUE Revenue Model	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
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>8%</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	1237.5	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
TOTAL REVENUE	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$9.3	\$43.7	\$158.7	\$229.6	\$335.1

Source: Company reports and PJC Estimates

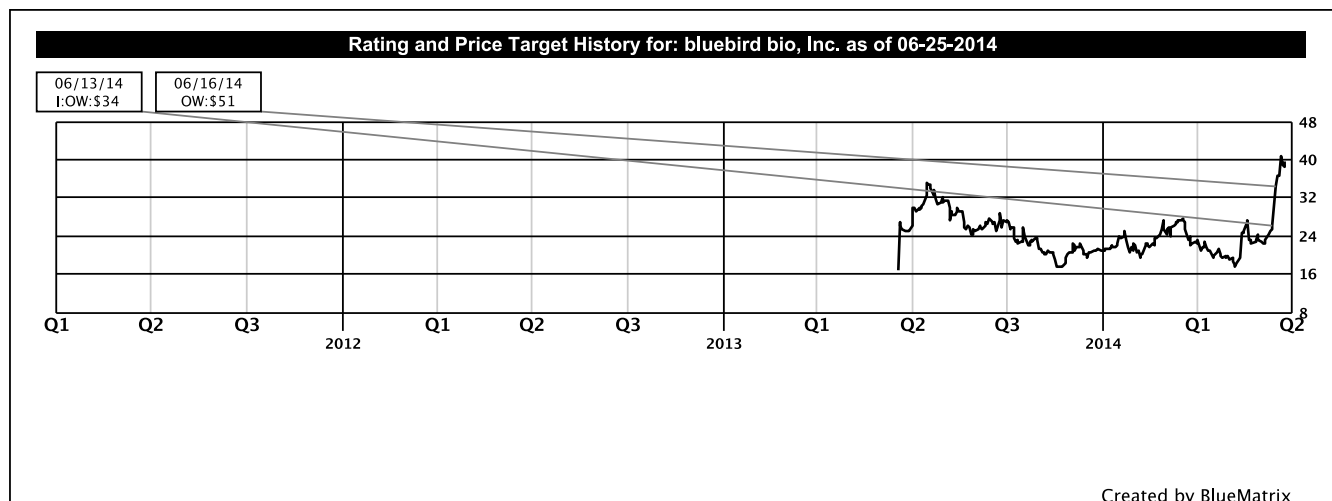
BLUE P&L (MM)	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
Collaboration revenue	0.1	19.8	24.3	20.0	20.0	20.0	23.0	25.0	25.0	25.0	25.0
Research and license fees	0.7	0.4	0.1	1.0	1.0	1.0	1.0	1.0	1.0	1.0	1.0
Product revenue	0.0	0.0	0.0	0.0	0.0	0.0	9.3	43.7	158.7	229.6	335.1
Other product-associated revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total Revenue	\$0.7	\$20.2	\$24.3	\$21.0	\$21.0	\$21.0	\$33.3	\$69.7	\$184.7	\$255.6	\$361.1
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	80.0
General and administrative	6.8	14.1	22.0	25.0	25.0	35.0	60.0	65.0	85.0	110.0	130.0
Total Operating Expenses	\$24.1	\$45.1	\$69.5	\$73.0	\$73.0	\$83.0	\$110.7	\$118.1	\$156.1	\$191.1	\$233.5
Operating Income (GAAP)	(\$23.3)	(\$24.9)	(\$45.2)	(\$52.0)	(\$52.0)	(\$62.0)	(\$77.3)	(\$48.4)	\$28.6	\$64.5	\$127.6
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
Pretax Income (loss)	(23.7)	(25.3)	(42.7)	(48.5)	(48.5)	(57.5)	(72.3)	(42.4)	34.6	71.5	135.6
<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>
Taxes	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	10.7	27.1
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	108.5
Diluted EPS (as reported/GAAP)	(\$13.79)	(\$2.02)	(\$1.74)	(\$1.84)	(\$1.62)	(\$1.83)	(\$2.02)	(\$1.14)	\$0.86	\$1.47	\$2.53
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

BLUE Cash Flow Statement	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
Net income	(4)	(25)	(43)	(49)	(49)	(58)	(72)	(42)	35	61	108
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
Net Cash Provided by Operating Activities	(21)	43.5	(43)	(47)	(48)	(59)	(69)	(28)	49	76	131
Capital expenditures	0	(9)	(4)	(5)	(4)	(4)	(4)	(4)	(4)	(4)	(4)
Free Cash Flow	(21)	35	(47)	(52)	(52)	(63)	(73)	(32)	45	72	127
Net Cash From Financing	60	106	1	2	75	2	100	2	2	2	3
Cash and Cash Equivalents	67	206	159	109	133	72	99	69	115	189	320

Sources: Company Reports and Piper Jaffray

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 T: Transferring Coverage
 D: Discontinuing Coverage
 S: Suspending Coverage
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 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
BUY [OW]	356	61.91	87	24.44
HOLD [N]	204	35.48	21	10.29
SELL [UW]	15	2.61	0	0.00

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Analyst Certification — Joshua E. Schimmer, MD, Sr Research Analyst

— Kristina N. Cibor, Research Analyst

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- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

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