

bluebird bio (BLUE)

Initial Beta-Thal Data De-risks LentiGlobin Program, Increasing PT to \$51 (from \$40)

- BLUE management hosted a call this morning detailing the results from the first two patients treated in the Phase I/II (HGB-205) study of LentiGlobin in beta-thal. Expanding upon the data made available over the weekend in an oral presentation at the European Hematology Association (EHA) Congress, management presented slides today demonstrating the consistent improvement in corrected beta-globin production over the initial months post-treatment (see figures on next page). As previously noted, the two patients were producing 4.2 and 6.6 g/dL of T87Q-corrected globin (36% and 65% of total hemoglobin produced, respectively) at 2 and 4.5 months post-treatment, superior to the 2.7 g/dL of corrected globin (about 30% of total hemoglobin) peak observed at 1.5 years following treatment with the prior-generation LentiGlobin vector in the best responder (Subject 3) from the Phase I/II LG001 study. Both patients in the HGB-205 study became transfusion-independent within two weeks following treatment, whereas Subject 3 did not achieve the same status until one year post-treatment (and has remained so for six years).
- This initial data gives us greater confidence in the LentiGlobin program, and we view it as a key inflection point for the company. The data presented gives us assurance that the clinical response seen in the two HGB-205 patients will exceed what has been observed in Subject 3 from LG001, including potentially life-long transfusion independence. We also expect a similar response to be observed in sickle-cell disease (SCD), considering the anti-sickling properties of the new LentiGlobin vector and the activity demonstrated in preclinical models of the disease. We expect the first SCD patient to be treated with LentiGlobin by the end of the year, either in the French HGB-205 study or the planned HGB-206 study in the US.
- We are reducing our discount rate for the LentiGlobin product from 35% to 30%, which raises our PT to \$51. We note that despite the run-up today in response to the data, BLUE still sports a market cap below Acceleron Pharma (XLRN, not covered), its clearest comparable. Acceleron is currently developing protein therapeutics (sotatercept and ACE-536) for the treatment of anemia in rare blood disorders.
- Reiterate OUTPERFORM and raising our price target to \$51 (from \$40). Our price target is derived from applying an 8X multiple to estimated 2019 revenues for Lenti-D (discounted 35% annually) and LentiGlobin (discounted 30% annually).

FYE Dec	2013A	2014E			2015E		
REV	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	\$1.1A	\$6.3A		\$6.3A	\$5.94E		5.9E
Q2 Jun	6.3A	6.3E		6.1E	6.3E		6.3E
Q3 Sep	6.4A	6.3E		6.1E	6.3E		6.3E
Q4 Dec	6.3A	6.3E		6.1E	6.3E		6.3E
Year*	\$20.2A	\$25.2E		\$24.7E	24.8E		24.2E
Change	5836%	25%			--		
EPS	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	(\$0.39)A	(\$0.44)A		(\$0.44)A	(\$0.55)E		(\$0.55)E
Q2 Jun	(0.19)A	(0.44)E		(0.45)E	(0.49)E		(0.49)E
Q3 Sep	(0.26)A	(0.44)E		(0.45)E	(0.50)E		(0.50)E
Q4 Dec	(0.34)A	(0.45)E		(0.45)E	(0.52)E		(0.52)E
Year*	(\$1.15)A	(\$1.77)E		(\$1.78)E	(2.06)E		(1.90)E
P/E	--	--			--		
Change	36%	-54%			--		

Consensus estimates are from Thomson First Call. * Numbers may not add up due to rounding.

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June 16, 2014

Price
\$35.02

Rating
OUTPERFORM

12-Month Price Target
\$51 (from \$40)

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Company Information

Shares Outst (M)	24.1
Market Cap (M)	\$845.7
52-Wk Range	\$17.03 - \$36.25
Book Value/sh	\$5.94
Cash/sh	\$7.97
Enterprise Value (M)	\$653.2
LT Debt/Cap %	0%
Cash Burn (M)	\$40.3

Company Description

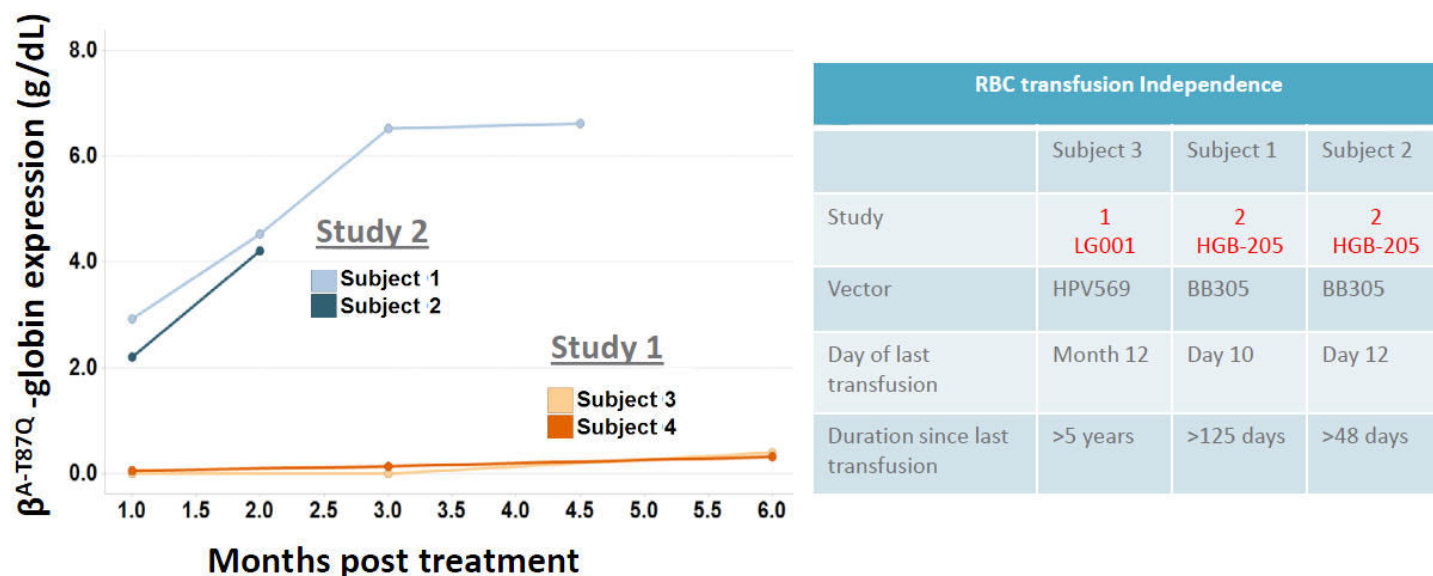
Bluebird bio is developing LENTI-D, a gene therapy in a Phase II/III trial for treating CCALD, and LENTIGLOBIN, a gene therapy in Phase I/II trials for treating beta-thalassemia and sickle cell disease. With Celgene, it is also developing CAR-T in oncology.



Source: Thomson Reuters

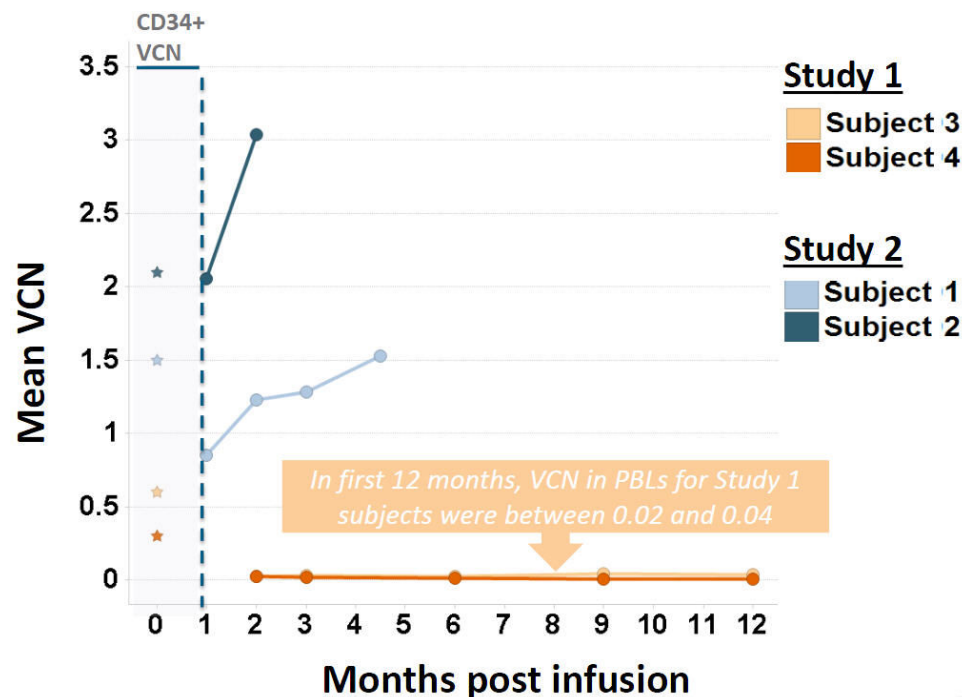
Risks to the achievement of our price target include failure to gain approval for products in development, failure to achieve sales estimates for any marketed product and failure to achieve earnings estimates.

Figure 1: Therapeutic Beta-Globin Expression in HGB-205 (Study 2) vs. LG001 (Study 1)



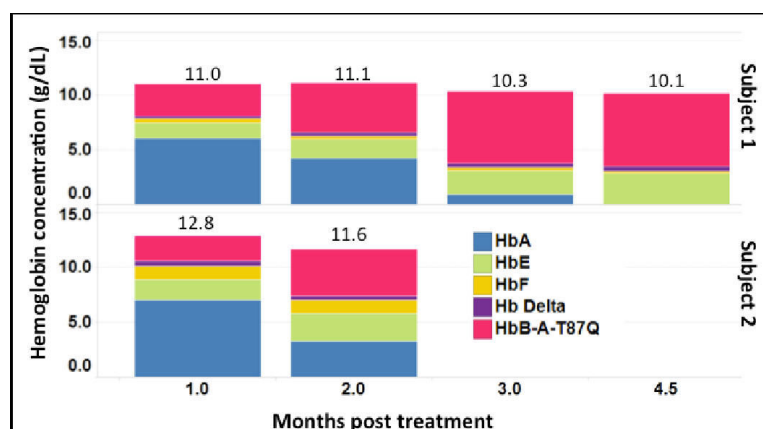
Source: Company data

Figure 2: Vector Copy Number Production in HGB-205 (Study 2) vs. LG001 (Study 1)

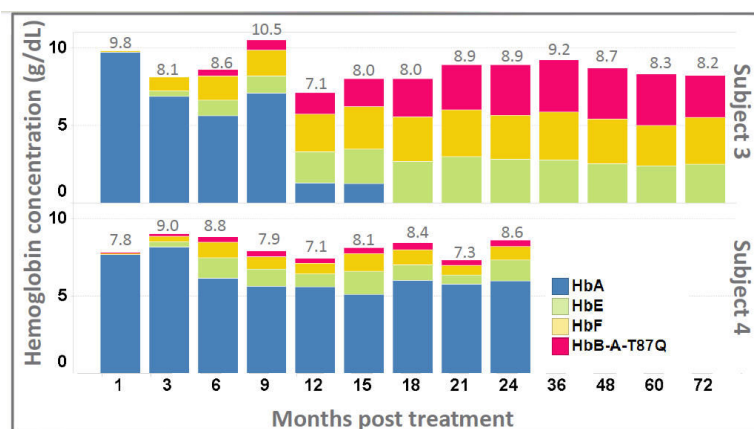


Source: Company data

Figure 3: Hemoglobin Concentration in HGB-205 (top) vs. LG001 (bottom)



- Subject 1: producing 6.6 g/dL of β^{A-T87Q} -globin at 4.5 months
- Subject 2: producing 4.2 g/dL of β^{A-T87Q} -globin at 2 months



- Subject 3: stable levels of β^{A-T87Q} -globin beginning at Month 18, **transfusion independent by Month 12**. Producing 2.7 g/dL of β^{A-T87Q} -globin at 6 years.
- Subject 4: minimal levels of β^{A-T87Q} -globin post-treatment, transfusion dependent. Producing 0.4 g/dL of β^{A-T87Q} -globin at 2 years.

Source: Company data

Investment Thesis

Bluebird bio is a gene therapy company focused on the treatment of rare genetic diseases, with two product candidates currently in development. Recent technical advances in gene therapy have revived strong interest in its curative potential and bluebird bio has made significant strides in the clinic. LENTI-D, its lead clinical product, is in a pivotal Phase II/III trial in patients with childhood adrenoleukodystrophy (ALD). Its other product in clinical development, LENTIGLOBIN, is in two Phase I/II trials in patients with the inherited hemoglobin disorders, β -thalassemia and sickle cell disease. Bluebird is also collaborating with Celgene to discover, develop and commercialize genetically modified T-cells, also called chimeric antigen receptor (CAR) cells, for the targeted destruction of cancer cells.



David M. Nierengarten, Ph.D.

6/16/2014

Bluebird Bio, Inc.

Annual Financial Results & Projections

(\$ in thousands except per share data)

Ticker: BLUE (Nasdaq)

	FY:12A	FY:13A	Q1	Q2	Q3	Q4	FY:14E	FY:15E	FY:16E	FY:17E	FY:18E	FY:19E	FY:20E
Revenue:													
Sales	0	0	0	0	0	0	0	0	0	116,154	115,864	689,872	1,401,048
Collaboration revenue	0	19,792	6,250	6,150	6,150	6,150	24,700	24,243	6,027	0	0	0	0
Grant, license fees and other revenue	340	389	85	150	150	150	535	600	1,050	1,200	1,200	1,200	1,200
Total Revenues	\$340	\$20,181	\$6,335	\$6,300	\$6,300	\$6,300	\$25,235	\$24,843	\$7,077	\$117,354	\$117,064	\$691,072	\$1,402,248
Cost and Expenses:													
Costs of goods sold	0	0	0	0	0	0	0	0	0	29,038	28,966	172,468	350,262
Research and Development	17,210	31,002	11,463	11,520	11,578	11,636	46,197	50,140	56,433	66,707	81,083	98,557	119,797
Sales, General and Administrative	6,846	14,126	5,540	5,568	5,596	5,624	22,327	29,712	45,864	54,679	54,624	163,685	298,809
Other operating expenses	0	0	0	0	0	0	0	0	0	0	0	0	8,000
Total Costs and Expenses	\$24,056	\$45,128	\$17,003	\$17,088	\$17,173	\$17,259	\$68,524	\$79,852	\$102,298	\$150,425	\$164,673	\$434,711	\$776,868
Operating Income (loss)	(23,716)	(24,947)	(10,668)	(10,788)	(10,873)	(10,959)	(43,289)	(55,009)	(95,221)	(33,071)	(47,609)	256,361	625,380
Net Interest Income (Expense)	5	29	59	192	173	156	581	669	561	619	444	433	5,103
Other income / (Expense)	41	(403)	0	0	0	0	0	0	0	0	0	0	0
Income Before Income Taxes	(23,670)	(25,321)	(10,609)	(10,596)	(10,700)	(10,803)	(42,708)	(54,340)	(94,660)	(32,452)	(47,165)	256,794	630,483
Net Income	(\$23,671)	(\$25,321)	(\$10,609)	(\$10,596)	(\$10,700)	(\$10,803)	(\$42,708)	(\$54,340)	(\$94,660)	(\$32,452)	(\$47,165)	\$243,954	\$447,661
GAAP Basic EPS with sFAS123	(1.81)	(1.15)	(0.44)	(0.44)	(0.44)	(0.45)	(1.77)	(2.06)	(3.39)	(1.08)	(1.56)	8.08	14.80
GAAP Diluted EPS with sFAS123	(1.81)	(1.15)	(0.44)	(0.44)	(0.44)	(0.45)	(1.77)	(2.06)	(3.39)	(1.08)	(1.56)	7.32	13.41
Shares outstanding	13,112	21,986	24,148	24,148	24,148	24,148	24,148	26,398	27,898	30,148	30,148	30,211	30,248
Cash Burn	(22,849)	(18,821)	(10,005)	(9,992)	(10,096)	(10,199)	(40,293)	(51,700)	(91,870)	(29,438)	(44,150)	246,975	450,685
Cash Balance	67,011	206,279	192,499	173,362	156,321	139,208	139,208	174,322	181,481	127,366	79,380	272,190	686,055

Analyst Biography

David is an Analyst covering stocks in the Biotechnology/Biopharmaceuticals/BioDefense sector. His prior sell-side research experience at Robert W. Baird & Co. covered biotechnology companies of all market capitalizations, with a focus on oncology and rare diseases.

David received his B.S. (Biochemistry) from the University of Wisconsin-Madison and Ph.D. (Molecular and Cell Biology) from the University of California-Berkeley.

David's Edge: David's early stage venture capital investing experience gives him a balanced perspective on developmental-stage biotechnology companies and their ultimate risk/reward potential. His experience on the other side of that equation in a clinical-stage, venture backed biotechnology company provides him with insights into corporate operations. The combination of experiences creates a focus on value creation in this event-driven space.

Analyst Certification

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Company	Disclosure
bluebird bio	1,3,4,5

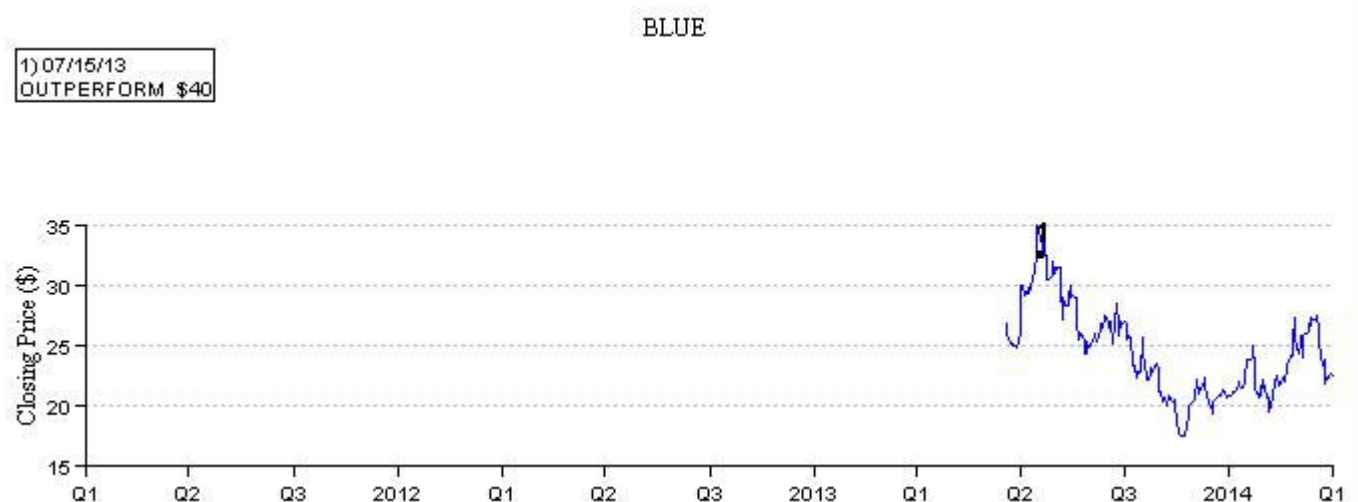
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* WS changed its rating system from (Strong Buy/Buy/Hold/Sell) to (Outperform/ Neutral/Underperform) on July 14, 2009.

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