

	Annual EPS	Annual Revenue	Rating/Target
Today's Changes	2013E \$(1.79) from \$(1.42)	2013E \$21.3M from \$21.0M	No changes
	2014E \$(0.71) from \$(0.63)	2014E \$27.3M, no change	

# bluebird bio

BLUE: NASDAO: US\$30.04

BUY

**Target: US\$45.00** 

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#### **COMPANY STATISTICS:**

52-week Range:	24.00 - 36.25
Market Cap (M):	US\$657
Avg. Daily Vol. (000s):	257
Shares Out (M):	21.869
Cash (M):	US\$67.01

# **EARNINGS SUMMARY:**

FYE Dec		2012A	2013E	2014E
EPS:		(13.79)	(1.79)	(0.71)
Revenue (M):	Q1		1.1A	
	Q2		6.3A	
	Q3		6.9	
	Q4		6.9	
Total		0.3	21.3	27.3
EPS:	Q1		(19.94)A	
	Q2		(2.13)A	
	Q3		(0.20)	
	Q4		(0.25)	
Total		(13.79)	(1.79)	(0.71)

### **SHARE PRICE PERFORMANCE:**



Source: Interactive Data Corporation

# **COMPANY DESCRIPTION:**

bluebird bio is a clinical-stage biotechnology company focused on gene therapy approaches to severe genetic and orphan disorders. The lead product is Lenti-D for CCALD. The second product is LentiGlobin for beta-thalassemia and sickle cell disease, and bluebird is also developing CAR T-based cancer therapies thorough a partnership with Celgene. bluebird is based in Cambridge, MA.

All amounts in US\$ unless otherwise noted.

# Life Sciences -- Biotechnology

# **Q2 EPS: CLINICAL TRIALS ON TRACK**

BLUE reported Q2 GAAP EPS of \$(2.13), below consensus of \$(0.35) and CGe of \$(0.92) primarily due to a higher share count following the IPO in June. We view BLUE as a leader in gene therapy and are optimistic on the odds for clinical success in both lead programs – particularly Lenti-D for childhood cerebral adrenoleukodystrophy (CCALD) given physician feedback and a P1/2 trial where three of four boys treated with an earlier version of Lenti-D demonstrated disease stabilization. The next key data catalysts will be interim data from one or both P1/2 LentiGlobin trials in late-2014.

- Clinical trial timeframes on track: BLUE reiterated guidance to initiate a P1/2 LentiGlobin trial in patients with beta-thalassemia the U.S. in mid-13 (recall, one P1/2 trial in the E.U. for patients with beta-thalassemia and sickle-cell disease has initiated). We model for pivotal beta-thalassemia trial initiation in mid-16, with launches in mid-20 (U.S.) and early-21 (E.U.) and peak LentiGlobin revenue recognized by BLUE of \$1.23B in 2026. BLUE will also initiate a pivotal P2/3 trial of Lenti-D for CCALD in late-13 we model for peak WW Lenti-D revenue recognized by BLUE of \$212M in 2028.
- Q2 financials: BLUE reported \$6.3M in revenue for Q2 (almost all due to recognized revenue from the CELG partnership), above consensus of \$4.5M and in-line with CGe of \$6.3M. BLUE exited Q2 with \$228.8M in cash and equivalents, including \$104.9M net from the initial public offering.

Canaccord Genuity is the global capital markets group of Canaccord Financial Inc. (CF: TSX | CF.: LSE)

The recommendations and opinions expressed in this research report accurately reflect the Investment Analyst's personal, independent and objective views about any and all the Designated Investments and Relevant Issuers discussed herein. For important information, please see the Important Disclosures section in the appendix of this document or visit Canaccord Genuity's <a href="Online Disclosure Database">Online Disclosure Database</a>.



Figure 1: Q2/13 variance table

(\$thousands, except per share data)									
		Jun		Jun	۷a	riance	Variance	Y/Y	Q/Q
	2	2Q13A	2	2Q13E		A-E	%	%	%
Revenue									
Lenti-D Total Revenue	\$	-	\$	-		-	n/a	n/a	n/a
Lenti-D - U.S.		-		-		-	n/a	n/a	n/a
Lenti-D - E.U.		-		-		-	n/a	n/a	n/a
LentiGlobin Total Revenue	\$	-	\$	-		-	n/a	n/a	n/a
LentiGlobin - U.S.		-		-		-	n/a	n/a	n/a
LentiGlobin - E.U.		-		-		-	n/a	n/a	n/a
Total other revenue	\$	6,334	\$	6,250		84	1%	7352%	462%
Collaboration revenue		6,249		6,250		(1)	0%	7252%	500%
Research and license fees		85		-		85	100%	n/a	0%
Grant revenue		-		-		-	n/a	n/a	n/a
Total Revenue	\$	6,334	\$	6,250	\$	84	1%	7352%	462%
COGS		-		-		-	n/a	n/a	n/a
Gross profit		6,334		6,250		84	1%	7352%	462%
Operating expense									
R&D (GAAP)		7,247		6,285		962	13%	116%	37%
SG&A (GAAP)		3,281		2,864		417	13%	147%	41%
Stock-based compensation		753		753		-	0%	n/a	8%
Total operating expense		10,528		9,149		1,379	13%	125%	38%
Operating income (loss)		(4,194)		(2,899)		(1,295)	31%	-9%	-35%
Other (expense) income, net		(389)		5		(394)	101%	-1151%	517%
Income Before Income Taxes		(4,583)		(2,894)		(1,689)	37%	0%	-30%
Income Tax Provision		-		-		-	n/a	n/a	n/a
Net loss applicable to common shareholders	\$	(4,583)	\$	(2,894)		(1,689)	37%	-22%	-30%
GAAP EPS (diluted)	\$	(2.13)	\$	(0.92)		(1.21)	57%	-91%	-89%
Weighted shares outstanding									
basic and diluted - GAAP	L	2,151		3,137		(986)	-46%	754%	555%

Source: Canaccord Genuity research, Company reports

Figure 2: Upcoming milestones

Product	Indication	Timing	Milestone
LentiGlobin	β-thalassemia	mid-13	Phase 1/2 U.S. trial (HGB-204) initation
Lenti-D	CCALD	4Q13	Pivotal Phase 2/3 U.S./E.U. pivotal trial (ALD-102) initation
LentiGlobin	SCD	2014	IND submission; U.S. Phase 1/2 trial initation
LentiGlobin	β-thalassemia / SCD	4Q14	Interim Phase 1/2 data
Lenti-D	CCALD	2H15	Interim Phase 2/3 data

Source: Canaccord Genuity research, Company reports



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Figure 3: Changes to model

(\$thousands, except per share data)	FY	13E	FY1	4E	FY1	15E	FY1	6E	FY1	7E	FY1	8E	FY1	9E	FY	20E	FY	21E	FY	22E	FY:	23E
	New	<u>Prior</u>	New	Prior	New	<u>Prior</u>	New	<u>Prior</u>	New	<u>Prior</u>	New	Prior										
Revenue																						
Lenti-D (CCALD) Total Revenue	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ 3,152	\$ 3,152	\$ 30,577	\$ 30,577	\$ 70,093	\$ 70,093	\$ 115,317	\$ 115,317	\$ 143,128	\$ 143,128	\$ 162,538	\$ 162,538
Lenti-D - U.S.	-	-	-	-	-		-	-	-	-	3,152	3,152	15,950	15,950	34,514	34,514	53,080	53,080	64,452	64,452	72,634	72,634
Lenti-D - E.U.	-	-	-	-	-	-	-	-	-	-	-		14,627	14,627	35,579	35,579	62,237	62,237	78,676	78,676	89,904	89,904
LentiGlobin (β-thalassemia) Total Revenue	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ 4,456	\$ 4,456	\$ 147,849	\$ 147,849	\$ 403,487	\$ 403,487	\$ 723,633	\$ 723,633
LentiGlobin - U.S.	-	-	-	-	-	-	-	-	-	-	-		-	-	4,456	4,456	19,409	19,409	41,421	41,421	62,027	62,027
LentiGlobin - E.U.	-	-	-	-	-	-	-	-	-	-	-		-		-	-	128,440	128,440	362,066	362,066	661,605	661,605
Total other revenue	\$ 21,294	\$ 21,040	\$ 27,325	\$ 27,325	\$ 27,325	\$ 27,325	\$ 7,533	\$ 7,533	\$ 1,163	\$ 1,163	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -
Total Revenue	\$ 21,294	\$ 21,040	\$ 27,325	\$ 27,325	\$ 27,325	\$ 27,325	\$ 7,533	\$ 7,533	\$ 1,163	\$ 1,163	\$ 3,152	\$ 3,152	\$ 30,577	\$ 30,577	\$ 74,549	\$ 74,549	\$ 263,166	\$ 263,166	\$ 546,615	\$ 546,615	\$ 886,171	\$ 886,171
COGS	-		-	-				-	- 1		788	788	6,727	6,727	14,910	14,910	47,370	47,370	98,391	98,391	159,511	159,511
Gross profit	21,294	21,040	27,325	27,325	27,325	27,325	7,533	7,533	1,163	1,163	2,364	2,364	23,850	23,850	59,639	59,639	215,796	215,796	448,224	448,224	726,660	726,660
Operating expense																						
R&D (GAAP)	28,482	26,110	29,015	28,083	30,289	29,533	31,503	31,503	33,593	33,593	35,605	35,605	37,504	37,504	39,481	39,481	41,502	41,502	44,689	43,550	47,590	45,503
SG&A (GAAP)	13,779	12,937	14,640	14,431	15,538	15,538	17,011	17,011	25,715	25,715	27,280	27,280	34,731	34,731	42,202	42,202	44,283	44,283	46,908	46,189	49,430	48,200
Stock-based compensation	3,174	3,174	3,623	3,623	4,231	4,231	4,805	4,805	5,580	5,580	6,253	6,253	7,080	7,080	8,101	8,101	9,111	9,111	10,034	10,034	11,003	11,003
Total operating expense	42,261	39,047	43,655	42,514	45,827	45,071	48,514	48,514	59,308	59,308	62,885	62,885	72,235	72,235	81,683	81,683	85,785	85,785	91,597	89,739	97,020	93,703
Operating income (loss)	(20,968)	(18,008)	(16,330)	(15,189)	(18,502)	(17,746)	(40,981)	(40,981)	(58,146)	(58,146)	(60,521)	(60,521)	(48,385)	(48,385)	(22,044)	(22,044)	130,011	130,011	356,627	358,485	629,640	632,957
Other (expense) income, net	(439)	(45)	6	6	4	4	7	6	10	10	8	8	6	6	5	5	12	12	38	37	91	91
Net gain (loss)	(21,407)	(18,053)	(16,324)	(15,183)	(18,498)	(17,742)	(40,974)	(40,975)	(58,136)	(58,136)	(60,513)	(60,514)	(48,379)	(48,379)	(22,038)	(22,039)	130,023	130,023	356,665	358,523	629,731	633,047
Income Tax Provision		` - '			· · ·	- 1	` - '		` - '	` - '	- 1	` - '			- '	` - '	-	-	42,800	43,023	201,514	202,575
Net loss applicable to common shareholders	\$ (21,407)	\$ (18,053)	\$ (16,324)	\$ (15,183)	\$ (18,498)	\$ (17,742)	\$ (40,974)	\$ (40,975)	\$ (58,136)	\$ (58,136)	\$ (60,513)	\$ (60,514)	\$ (48,379)	\$ (48,379)	\$ (22,038)	\$ (22,039)	\$ 130,023	\$ 130,023	\$ 313,865	\$ 315,500	\$ 428,217	\$ 430,472
GAAP EPS (diluted)	\$ (1.79)	\$ (1.42)	\$ (0.71)	\$ (0.63)	\$ (0.80)	\$ (0.73)	\$ (1.58)	\$ (1.52)	\$ (2.03)	\$ (1.96)	\$ (2.09)	\$ (2.02)	\$ (1.66)	\$ (1.60)	\$ (0.75)	\$ (0.72)	\$ 4.21	\$ 4.07	\$ 9.84	\$ 9.57	\$ 12.73	\$ 12.39
Weighted shares outstanding																						
basic and diluted	11,936	12,679	22,915	23,921	23,145	24,160	25,876	26,902	28,635	29,671	28,921	29,968	29,210	30,267	29,502	30,570	30,867	31,946	31,889	32,978	33,635	34,734

Source: Canaccord Genuity research, Company reports



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Figure 4: BLUE Income statement

(\$thousands, except per share data)

	FY	FY 2012A	Mar 1Q13A	Jun 2Q13A	Sep 3Q13E	Dec 4Q13E	FY	FY	FY	FY	FY	FY	FY	FY	FY	FY	FY
Revenue	2011A	2012A	TQT3A	ZQT3A	JUIJE	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Lenti-D (CCALD) Total Revenue	\$ -	s -	_	_	_		e .	e _	s -	e _	<b>s</b> -	\$ 3,152	\$ 30,577	\$ 70,093	\$ 115,317	\$ 143,128	\$ 162,538
Lenti-D - U.S.	-	•	_	_	_	-	-	φ -	φ -	φ -	φ - -	3,152	15,950	34,514	53,080	64,452	72,634
Lenti-D - E.U.	_	_	_	_	_	_	_		_	_			14,627	35,579	62,237	78,676	89.904
LentiGlobin (β-thalassemia) Total Revenue	s -	s -	-	-	_	_	s -	s -	<b>s</b> -	s -	<b>s</b> -	s -	\$ -	\$ 4,456	\$ 147.849	\$ 403.487	\$ 723.633
LentiGlobin - U.S.			-	-	-	_						_		4,456	19,409	41,421	62,027
LentiGlobin - E.U.	-	-	-	-	-	-	-	-	-	-		-	-	-	128,440	362,066	661,605
Total other revenue	\$ 882	\$ 340	1,127	6,334	6,916	6,916	\$ 21,294	\$ 27,325	\$ 27,325	\$ 7,533	\$ 1,163	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -
Total Revenue	\$ 882	\$ 340	\$ 1,127	\$ 6,334	\$ 6,916	\$ 6,916	\$ 21,294	\$ 27,325	\$ 27,325	\$ 7,533	\$ 1,163	\$ 3,152	\$ 30,577	\$ 74,549	\$ 263,166	\$ 546,615	\$ 886,171
COGS			_		_							788	6,727	14,910	47,370	98,391	159.511
Gross profit	882	340	1,127	6,334	6,916	6,916	21,294	27,325	27,325	7,533	1,163	2,364	23,850	59,639	215,796	448,224	726,660
Gloss profit	002	340	1,127	0,334	0,910	0,910	21,294	21,323	21,323	7,555	1,103	2,304	23,630	59,659	215,790	440,224	720,000
Operating expense							_										
R&D (GAAP)	11,409	17,210	5,284	7,247	7,701	8,250	28,482	29,015	30,289	31,503	33,593	35,605	37,504	39,481	41,502	44,689	47,590
SG&A (GAAP)	4,615	6,846	2,324	3,281	3,765	4,409	13,779	14,640	15,538	17,011	25,715	27,280	34,731	42,202	44,283	46,908	49,430
Total operating expense	16,024	24,056	7,608	10,528	11,466	12,659	42,261	43,655	45,827	48,514	59,308	62,885	72,235	81,683	85,785	91,597	97,020
Operating income (loss)	(15,142)	(23,716)	(6,481)	(4,194)	(4,550)	(5,743)	(20,968)	(16,330)	(18,502)	(40,981)	(58,146)	(60,521)	(48,385)	(22,044)	130,011	356,627	629,640
Other (expense) income, net	(456)	46	(63)	(389)	7	6	(439)	6	4	7	10	8	6	5	12	38	91
		(00.070)			(4.540)	(5.707)		(40.004)	(40,400)	(40.074)		(00.540)	(40.070)	(00.000)			
Net gain (loss) Income Tax Provision	(15,598)	(23,670)	(6,544)	(4,583)	(4,543)	(5,737)	(21,407)	(16,324)	(18,498)	(40,974)	(58,136)	(60,513)	(48,379)	(22,038)	130,023	<b>356,665</b> 42,800	<b>629,731</b> 201,514
Net loss applicable to common shareholders	\$ (20,591)	\$ (3,613)	\$ (6,544)	\$ (4,583)	\$ (4,543)	\$ (5,737)	\$ (21,407)	\$ (16,324)	\$ (18,498)	\$ (40,974)	\$ (58,136)	\$ (60,513)	\$ (48,379)	\$ (22,038)	\$ 130.023	\$ 313,865	\$ 428.217
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GAAP EPS (diluted)	\$ (171.59)	\$ (13.79)	\$ (19.94)	\$ (2.13)	\$ (0.20)	\$ (0.25)	\$ (1.79)	\$ (0.71)	\$ (0.80)	\$ (1.58)	\$ (2.03)	\$ (2.09)	\$ (1.66)	\$ (0.75)	\$ 4.21	\$ 9.84	\$ 12.73
Weighted shares outstanding																	
Weighted shares outstanding basic and diluted	120	262	328	2,151	22,576	22,689	11,936	22,915	23,145	25,876	28,635	28,921	29,210	29,502	30,867	31,889	33,635
ŭ ü	120	262	328	2,151	22,576	22,689	11,936	22,915	23,145	25,876	28,635	28,921	29,210	29,502	30,867	31,889	33,635
basic and diluted  Margin Analysis:	120	262	328	2,151	22,576	22,689	11,936	22,915	·	25,876		_					
basic and diluted  Marqin Analysis: Cost of product sales	120 nm	nm	nm	nm	nm	nm	nm	22,915 nm	nm	nm	nm	25%	22%	20%	18%	18%	18%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin		nm nm	nm nm	nm nm	nm nm	nm nm	nm nm	nm nm	nm nm	nm nm	nm nm	25% 75%	22% 78%	20% 80%	18% 82%	18% 82%	18% 82%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP)	nm nm nm	nm nm 469%	nm nm 469%	nm nm 114%	nm nm 111%	nm nm 119%	nm nm 134%	nm nm 106%	nm nm 111%	nm nm 418%	nm nm 2890%	25% 75% 1130%	22% 78% 123%	20% 80% 53%	18% 82% 16%	18% 82% 8%	18% 82% 5%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP)	nm nm nm	nm nm 469% 206%	nm nm 469% 206%	nm nm 114% 52%	nm nm 111% 54%	nm nm 119% 64%	nm nm 134% 65%	nm nm 106% 54%	nm nm 111% 57%	nm nm 418% 226%	nm nm 2890% 2212%	25% 75% 1130% 866%	22% 78% 123% 114%	20% 80% 53% 57%	18% 82% 16% 17%	18% 82% 8% 9%	18% 82% 5% 6%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP) Stock-based compensation expense	nm nm nm	nm nm 469%	nm nm 469%	nm nm 114% 52% 12%	nm nm 111% 54% 12%	nm nm 119% 64% 13%	nm nm 134% 65% 15%	nm nm 106% 54% 13%	nm nm 111% 57% 15%	nm nm 418% 226% 64%	nm nm 2890% 2212% 480%	25% 75% 1130% 866% 198%	22% 78% 123% 114% 23%	20% 80% 53% 57% 11%	18% 82% 16% 17% 3%	18% 82% 8% 9% 2%	18% 82% 5% 6% 1%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP) Stock-based compensation expense Total operating expense	nm nm nm	nm nm 469% 206%	nm nm 469% 206%	nm nm 114% 52%	nm nm 111% 54%	nm nm 119% 64%	nm nm 134% 65%	nm nm 106% 54%	nm nm 111% 57%	nm nm 418% 226%	nm nm 2890% 2212%	25% 75% 1130% 866%	22% 78% 123% 114%	20% 80% 53% 57%	18% 82% 16% 17% 3% 33%	18% 82% 8% 9% 2% 17%	18% 82% 5% 6% 1% 11%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP) Stock-based compensation expense Total operating expense Operating margin	nm nm nm nm	nm nm 469% 206% 18% nm	nm nm 469% 206% 62% nm nm	nm nm 114% 52% 12% 166% nm	nm nm 111% 54% 12% 166%	nm nm 119% 64% 13% 183%	nm nm 134% 65% 15% 198%	nm nm 106% 54% 13% 160%	nm nm 111% 57% 15% 168%	nm nm 418% 226% 64% 644%	nm nm 2890% 2212% 480% 5102% nm	25% 75% 1130% 866% 198% 1995%	22% 78% 123% 114% 23% 236%	20% 80% 53% 57% 11% 110%	18% 82% 16% 17% 3% 33% 49%	18% 82% 8% 9% 2% 17% 65%	18% 82% 5% 6% 1% 11% 71%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) Stock-based compensation expense Total operating expense Operating margin Income tax provision	nm nm nm nm nm	nm nm 469% 206% 18% nm	nm nm 469% 206% 62% nm	nm nm 114% 52% 12% 166% nm 0%	nm nm 111% 54% 12% 166% nm	nm nm 119% 64% 13% 183% nm	nm nm 134% 65% 15% 198% nm	nm nm 106% 54% 13% 160% nm	nm nm 111% 57% 15% 168% nm	nm nm 418% 226% 64% 644% nm 0%	nm nm 2890% 2212% 480% 5102% nm	25% 75% 1130% 866% 198% 1995% nm	22% 78% 123% 114% 236% 236% nm	20% 80% 53% 57% 11% 110% nm	18% 82% 16% 17% 3% 33% 49%	18% 82% 8% 9% 2% 17% 65%	18% 82% 5% 6% 1% 11% 71% 32%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP) Stock-based compensation expense Total operating expense Operating margin	nm nm nm nm nm	nm nm 469% 206% 18% nm	nm nm 469% 206% 62% nm nm	nm nm 114% 52% 12% 166% nm	nm nm 111% 54% 12% 166%	nm nm 119% 64% 13% 183%	nm nm 134% 65% 15% 198%	nm nm 106% 54% 13% 160%	nm nm 111% 57% 15% 168%	nm nm 418% 226% 64% 644%	nm nm 2890% 2212% 480% 5102% nm	25% 75% 1130% 866% 198% 1995%	22% 78% 123% 114% 23% 236%	20% 80% 53% 57% 11% 110%	18% 82% 16% 17% 3% 33% 49%	18% 82% 8% 9% 2% 17% 65%	18% 82% 5% 6% 1% 11% 71%
basic and diluted  Margin Analysis: Cost of product sales Product gross margin R&D (GAAP) SG&A (GAAP) Stock-based compensation expense Total operating expense Operating margin Income tax provision Net margin (GAAP)	nm nm nm nm nm nm	nm nm 469% 206% 18% nm nm	nm nm 469% 206% 62% nm nm	nm nm 114% 52% 12% 166% nm 0%	nm nm 111% 54% 12% 166% nm	nm nm 119% 64% 13% 183% nm	nm nm 134% 65% 15% 198% nm	nm nm 106% 54% 13% 160% nm	nm nm 111% 57% 15% 168% nm	nm nm 418% 226% 64% 644% nm 0%	nm nm 2890% 2212% 480% 5102% nm	25% 75% 1130% 866% 198% 1995% nm	22% 78% 123% 114% 236% 236% nm	20% 80% 53% 57% 11% 110% nm	18% 82% 16% 17% 3% 33% 49%	18% 82% 8% 9% 2% 17% 65%	18% 82% 5% 6% 1% 11% 71% 32%
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Source: Canaccord Genuity research, Company reports



# **Valuation**

We arrive at our 12-month price target of \$45 via averaging two valuation methods: 1) a sum-of-the-parts discounted cash flow analysis equating to \$46 a share which ascribes \$14/share from Lenti-D, \$23/share for LentiGlobin and \$8/share in cash, with the following assumptions: we assign Lenti-D a 75% chance of success and LentiGlobin a 30% chance of success and we assign a WACC of 10% and a 1% terminal growth rate; and 2) a discounted EPS equating to \$45/share, applying a 35x multiple to our FY22 fully diluted GAAP EPS estimate of \$9.84, discounted back to mid-14 at 27%.

#### Investment risks

The primary risks for bluebird include the following:

- 1. Lenti-D clinical development risk: efficacy will the Phase 2/3 trial demonstrate efficacy that compares favorably against the natural history trial (we note that the primary endpoint of no major functional disabilities is stringent and the new vector used in this trial has not been evaluated in the clinic; and safety --will a safety signal emerge (particularly leukemia or pre-leukemic clonal expansion)?
- 2. LentiGlobin clinical development risk: efficacy -- will the two Phase 1/2 trials demonstrate efficacy in beta-thalassemia patients (particularly sufficient hemoglobin for patients to become transfusion independent) and safety?
- 3. Commercial risk, including the possibility that Lenti-D and LentiGlobin do not achieve the peak commercial revenue estimates in our model (due to patient identification, market size, penetration rates, and/or pricing/reimbursement -- particularly given the anticipated price of \$1.5M+).
- 4. Regulatory risk: including failure to secure U.S. and E.U. approval for both Lenti-D and LentiGlobin.
- 5. Product competition, for Lenti-D, advances in hematopoietic stem cell transplant using non-related donors or other techniques to prevent demyelination or development of competing gene therapy techniques; for LentiGlobin, other gene therapy approaches or advances in the ability to upregulate the fetal gamma globin gene.
- 6. Financing risk -- we model for one equity offering (\$200M; 5M shares @ \$40/share) in mid-2016.



# **APPENDIX: IMPORTANT DISCLOSURES**

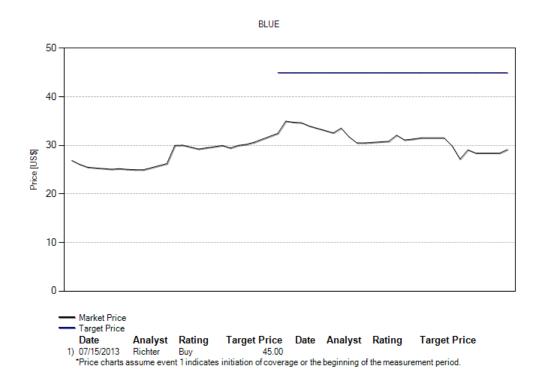
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**Site Visit:** 

An analyst has not visited the issuer's material operations.

#### Price Chart:\*



# **Distribution of Ratings:** Global Stock Ratings

(as of 28 June 2013)

	Coverage U	Jniverse	
Rating	#	%	IB Clients %
Buy	568	59.1%	36.6%
Speculative Buy	58	6.0%	60.3%
Hold	288	30.0%	11.1%
Sell	47	4.9%	6.4%
_	964*	100.0%	

<sup>\*</sup>Total includes stocks that are Under Review

# Canaccord Genuity Ratings System:

**BUY:** The stock is expected to generate risk-adjusted returns of over 10% during the next 12 months. **HOLD:** The stock is expected to generate risk-adjusted returns of 0-10% during the next 12 months. **SELL:** The stock is expected to generate negative risk-adjusted returns during the next 12 months. **NOT RATED:** Canaccord Genuity does not provide research coverage of the relevant issuer.

"Risk-adjusted return" refers to the expected return in relation to the amount of risk associated with the designated investment or the relevant issuer.

# **Risk Qualifier:**

**SPECULATIVE:** Stocks bear significantly higher risk that typically cannot be valued by normal fundamental criteria. Investments in the stock may result in material loss.



#### Canaccord Genuity Research Disclosures as of 14 August 2013

Company	Disclosure
bluebird bio	1A, 2, 3, 5, 7

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  - B. non-investment banking securities-related services.
  - C. non-securities related services.
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