COWEN
AND COMPANY

Biotechnology

bluebird bio

Equity Research

Earnings Update

May 14, 2014

Price: $19.42 (05/13/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: BLUE
52-Week Range:	$36.25 - 17.00
Market Cap (MM):	$476.0
Net Debt (MM):	$(206.3)
Cash/Share:	$8.62
Dil. Shares Out (MM):	24.1
Enterprise Value (MM):	$269.7
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$6.34
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	$(19.59)	$(0.44)A	-
Prior Q1	*$(0.41)*	*$(0.28)*	*-*
Q2	$(2.13)	$(0.45)	-
Prior Q2	*-*	*$(0.28)*	*-*
Q3	$(0.26)	$(0.46)	-
Prior Q3	*-*	*$(0.29)*	*-*
Q4	$(0.34)	$(0.47)	-
Prior Q4	*$(0.28)*	*$(0.29)*	*-*
Year	$(2.02)	$(1.81)	$(1.80)
Prior Year	*$(1.46)*	*$(1.15)*	*$(1.05)*
P/E	NM	NM	NM
Consensus EPS	$(1.52)	$(1.39)	$(1.46)
Prior Year	*-*	*$(1.52)*	*$(1.26)*

Consensus source: Thomson Reuters

Revenue (MM)			
Year	$20.2	$24.3	$26.0
Prior Year	*$19.8*	*$24.0*	*-*
EV/S	13.4x	11.1x	10.4x

Reports Q1, Initial Beta-Thalassemia Data In June

The Cowen Insight

bluebird bio provided a financial update and announced that preliminary results from LentiGlobin's Phase I/II trial in beta-thalassemia patients will be presented at the EHA meeting June 12-15. bluebird bio remains well financed, and we expect shares to outperform as its gene therapy candidates progress through clinical development.

Early Phase I/II HGB-205 Data To Be Presented Well Ahead Of Our Expectations

BLUE's Phase I/II HGB-205 trial is employing a modified LentiGlobin vector (for ex-vivo transduction of HSCs) that could be more efficient than the vector employed in a prior French academic study. Recall the first patient in this seven-patient trial was transplanted in December 2013, and we had anticipated a first look at data in Q4:14. Last night, bluebird announced some early data will be presented at the 19th EHA meeting in Milan, June 12-15. We believe earlier than anticipated results bode well for LentiGlobin's ability to demonstrate early signs of activity in this trial, and provide further proof of concept for this mechanism. We would expect the presentation to include results on just a few patients, including changes in hemoglobin levels and in vivo characterization of LentiGlobin's transduction efficiency (# of cells carrying one or more copies of the transgene). EHA abstracts will be available online on Wednesday, May 21st.

Furthering The Understanding Of CCALD Patients Treated By Allogeneic HSCT

During Q1, bluebird filed an IND amendment to conduct a non-interventional observational study (ALD-103) of CCALD patients treated by *allogeneic* HSCT. Via this trial, bluebird intends to gain further insight on CCALD patient population treated with *allogeneic* HSCT, including information on GvHD complications and outcomes. Future Lenti-D trials will benefit from this additional knowledge.

R&D Expenses Increase As Pipeline Ramps

bluebird reported a Q1 net loss of $10.6MM vs. our estimate of $6.9MM. The difference was related to increase in R&D spending as beta-thalassemia and CCALD studies progress. bluebird ended Q1:14 with $193M in cash, enough to fund operations through 2016 assuming no new business development activities.

At A Glance

Our Investment Thesis

bluebird bio is advancing novel gene therapies for the treatment of severe, orphan diseases. Proof-of-concept efficacy has been demonstrated in early studies. We are optimistic that Lenti-D (for the treatment of CCALD) and LentiGlobin (for the treatment of beta-thalassemia) could represent meaningful advances in areas of unmet need. Our valuation analysis suggests shares may be 40-45% undervalued with potential upside from LentiGlobin in sickle cell disease, a CAR T-cell collaboration with CELG, or future pipeline programs. Following a $100MM+ IPO completed in June 2013. bluebird has nearly $200MM in cash, enough to fund operations for several years assuming no new business development activity. We expect multiple value creating milestones to drive stock outperformance.

Forthcoming Catalysts

- Preliminary results from Phase I/II HGB-205 study at the 19th EHA meeting (June13-16, 2014)
- Initiate U.S. trial of LentiGlobin in sickle cell patients in 2014
- Interim data from E.U. LentiGlobin Study HGB-205 in beta-thalassemia/sickle cell patients in H2:14
- Initial data from U.S. LentiGlobin study HGB-204 in beta-thalassemia patients in H2:14

Base Case Assumptions

- Lenti-D for CCALD continues to progress through clinical development
- LentiGlobin for beta-Thalassemia continues to progress through clinical development
- Gene Therapy platform yields new potential therapies

Upside Scenario

- LentiGlobin demonstrates efficacy in sickle cell disease
- CELG collaboration in CAR-T cells in cancer treatments demonstrates activity

Downside Scenario

- Lenti-D for CCALD shows safety issues and/or is not suitable for development
- LentiGlobin for beta-Thalassemia fails to show efficacy in clinical development
- Gene Therapy platform is not productive

Price Performance

Source: Bloomberg

Company Description

bluebird bio seeks to provide transformative one-time gene therapy-based treatments to patients with severe orphan diseases. The company has assembled a leading gene therapy platform that has been industrialized to the point where it is capable of delivering consistent, high-quality gene therapies at scale. bluebird bio is directing its gene therapies toward indications of high unmet need where the likelihood of clinical, regulatory and commercial success in greatest. The company has generated proof-of-concept data in two genetic conditions: childhood cerebral adrenoleukodystrophy (CCALD) and β-Thalassemia. bluebird retains full ownership to these programs. A separate, early-stage collaboration with Celgene based upon chimeric antigen receptor (CAR) T cells aimed at cancer is ongoing. bluebird bio raised $100MM+ with an IPO in June 2013.

Analyst Top Picks

	Ticker	Price (05/13/2014)	Price Target	Rating
Sunesis Pharmaceuticals	SNSS	$4.76	$NA	Outperform
Relypsa, Inc	RLYP	$22.70	$NA	Outperform
Ultragenyx	RARE	$33.44	$NA	Outperform

Investment Thesis

bluebird bio seeks to provide transformative one-time gene therapy-based treatments to patients with severe orphan diseases. The company has assembled a leading gene therapy platform (novel vectors, transduction protocols, manufacturing processes) that has been industrialized to the point where it is capable of delivering consistent, high-quality gene therapies at scale. bluebird bio is directing its gene therapies toward indications of high unmet need where the likelihood of clinical, regulatory and commercial success in greatest. The company has generated proof-of-concept data in two genetic conditions: childhood cerebral adrenoleukodystrophy (CCALD), an X-linked disorder of progressive neurodegenerative decline, and b-Thalassemia, an autosomal recessive disease of red blood cell dysfunction characterized by severe anemia. These programs are de-risked by early clinical proof of concept in academic studies, and bluebird retains full ownership to these programs. In October, bluebird enrolled the first patients in a potentially pivotal trial in CCALD. A separate, early-stage collaboration with Celgene based upon chimeric antigen receptor (CAR) T cells is aimed at cancer. Following a $100MM+ IPO completed in June 2013, bluebird has around $200MM in cash, enough to fund operations for 5+ years assuming no new business development activity. We expect multiple value creating milestones to drive stock outperformance.

Bluebird Bio Upcoming Milestones

Milestone	Timing
Presentation of preliminary results from Phase I/II HGB-205 study at the 19th EHA meeting	June 12-15
Initiate U.S. trial of LentiGlobin in sickle cell patients	2014
Interim data from E.U. LentiGlobin Study HGB-205 in seven β-thalassemia / sickle cell patients	Late 2014
Initial data from U.S. LentiGlobin Study HGB-204 in up to 15 β-thalassemia patients	Late 2014
Possible initiation of Lenti-D trials in adult CALD patients	2015
Likely completion of enrollment (n=12-15) in pivotal CCALD trial	2015
Possible first IND in CAR T-cell oncology collaboration with Celgene	2015
Possible initiation of a pivotal trial on LentiGlobin	2015

Source: Cowen and Company

bluebird bio Quarterly P&L Model ($MM)

	Q1:13A	Q2:13A	Q3:13A	Q4:13A	2013A	Q1:14A	Q2:14E	Q3:14E	Q4:14E	2014E
Lenti-D Revenue										
LentiGlobin Revenue										
Collaborative and Grant Revenue	1.1	6.3	6.4	6.3	20.2	6.3	6.0	6.0	6.0	24.3
Total Revenue	**1.1**	**6.3**	**6.4**	**6.3**	**20.2**	**6.3**	**6.0**	**6.0**	**6.0**	**24.3**
Y/Y growth										
COGS	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
R&D	5.3	7.2	8.7	9.8	31.0	11.5	11.5	11.6	11.8	46.4
SG&A	2.3	3.3	3.8	4.7	14.1	5.5	5.5	5.7	5.8	22.5
Total Expenses	**7.6**	**10.5**	**12.5**	**14.4**	**45.1**	**17.0**	**17.0**	**17.3**	**17.6**	**68.9**
Operating Income/Loss	**(6.5)**	**(4.2)**	**(6.2)**	**(8.1)**	**(24.9)**	**(10.7)**	**(11.0)**	**(11.3)**	**(11.6)**	**(44.6)**
Non-Operating Income	(0.1)	(0.4)	0.0	0.0	(0.4)	0.1	0.0	0.0	0.0	0.1
Pre-tax Income/Loss	**(6.5)**	**(4.6)**	**(6.1)**	**(8.1)**	**(25.3)**	**(10.6)**	**(11.0)**	**(11.3)**	**(11.6)**	**(44.5)**
Tax rate (%)	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*
Provision for income taxes	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (Loss) From Operations	**(6.5)**	**(4.6)**	**(6.1)**	**(8.1)**	**(25.3)**	**(10.6)**	**(11.0)**	**(11.3)**	**(11.6)**	**(44.5)**
GAAP EPS	**($19.59)**	**($2.13)**	**($0.26)**	**($0.34)**	**($2.02)**	**($0.44)**	**($0.45)**	**($0.46)**	**($0.47)**	**($1.81)**
Diluted Shares	0.3	2.2	23.6	24.1	12.6	24.2	24.6	24.8	24.9	24.6

Source: Cowen and Company

bluebird bio Annual P&L Model ($MM)

	2013A	2014E	2015E	2016E	2017E	2018E
Lenti-D Revenue	0.0	0.0	0.0	0.0	0.0	0.0
LentiGlobin Revenue	0.0	0.0	0.0	0.0	0.0	0.0
Collaborative and Grant Revenue	20.2	24.3	26.0	28.0	30.0	32.0
Total Revenue	**20.2**	**24.3**	**26.0**	**28.0**	**30.0**	**32.0**
Y/Y growth		*0%*	*7%*	*8%*	*7%*	*7%*
COGS	0.0	0.0	0.0	0.0	0.0	0.0
R&D	31.0	46.4	50.0	53.0	55.0	59.0
SG&A	14.1	22.5	24.0	27.0	29.0	35.0
Total Expenses	**45.1**	**68.9**	**74.0**	**80.0**	**84.0**	**94.0**
Operating Income/Loss	**(24.9)**	**(44.6)**	**(48.0)**	**(52.0)**	**(54.0)**	**(62.0)**
Non-Operating Income	(0.4)	0.1	0.0	0.0	0.0	0.0
Pre-tax Income/Loss	**(25.3)**	**(44.5)**	**(48.0)**	**(52.0)**	**(54.0)**	**(62.0)**
Tax rate (%)	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*
Provision for income taxes	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (Loss) From Operations	**(25.3)**	**(44.5)**	**(48.0)**	**(52.0)**	**(54.0)**	**(62.0)**
GAAP EPS	**($2.02)**	**($1.81)**	**($1.80)**	**($1.85)**	**($1.80)**	**($2.00)**
Diluted Shares	12.6	24.6	26.7	28.1	30.0	31.0

Source: Cowen and Company

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:

In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:

There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

bluebird bio has no approved products and limited revenue. The company may need to raise additional capital from the public markets prior to turning profitable. bluebird's two lead candidates (Lenti-D and LentiGlobin) are gene therapies with little clinical trial experience. Each faces a number of clinical, regulatory, and commercial hurdles prior to becoming successful.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
BLUE	bluebird bio
RLYP	Relypsa, Inc
SNSS	Sunesis Pharmaceuticals
RARE	Ultragenyx

Analyst Certification

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Sunesis Pharmaceuticals is or was in the past 12 months a client of Cowen and Company, LLC; during the past 12 months, Cowen and Company, LLC provided Non-Security services.

bluebird bio, Ultragenyx, Relypsa, Inc and Sunesis Pharmaceuticals have been client(s) of Cowen and Company, LLC in the past 12 months.

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bluebird bio, Ultragenyx, Relypsa, Inc and Sunesis Pharmaceuticals is or was in the past 12 months a client of Cowen and Company, LLC; during the past 12 months, Cowen and Company, LLC provided IB services.

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Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

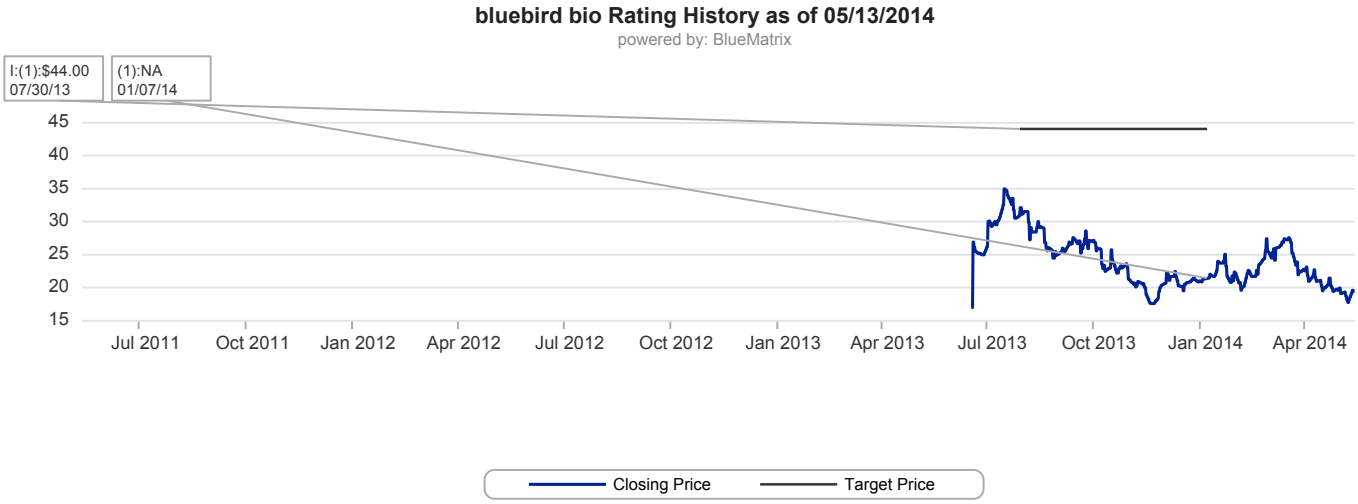
Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	407	57.08%	85	20.88%
Hold (b)	288	40.39%	8	2.78%
Sell (c)	18	2.52%	1	5.56%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.



Ultragenyx Rating History as of 05/13/2014
powered by: BlueMatrix

Relypsa, Inc Rating History as of 05/13/2014
powered by: BlueMatrix

Sunesis Pharmaceuticals Rating History as of 05/13/2014
powered by: BlueMatrix

Rating Change - 2/21/2006 - Outperform Rating

Legend for Price Chart:

I = Initation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | $xx = Price Target | NA = Not Available | S=Suspended

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