COMPANY UPDATE
Biotechnology
November 19, 2013

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# BLUEBIRD BIO INC (NASDAQ:BLUE)

# Update Following Meetings With Management

**Our meetings with senior management indicated that bluebird's trials in childhood cerebral adrenoleukodystrophy (CCALD), beta thalassemia, and sickle cell disease are on track. We expect management will continue to advance its leadership position within gene therapy.**

**Recommendation**

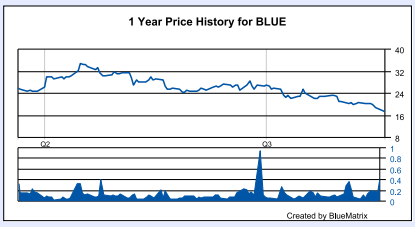
| | |
|---|---|
| Rating: | Outperform |
| Price Target (in $): | $44.00 |
| Expected Return: | 148.2% |
| Dividend: | NA |
| Enterprise Value (MM): | $412.0 |

**Stock Statistics as of 11/18/2013 (in $)**

| | |
|---|---|
| Price: | $17.73 |
| 52W Range: | $36.25-$17.00 |
| Shares Out (MM): | 23.6 |
| Market Cap (MM): | $421.6 |
| Net Debt (MM): | $0.0 |

**Fundamentals**

| | |
|---|---|
| Earnings Per Share ('12A) | *$(1.83)* |
| Earnings Per Share ('13E) | *$(1.46)* |
| Earnings Per Share ('14E) | *$(1.15)* |
| Revenue (MM) ('12A) | 0.3 |
| Revenue (MM) ('13E) | 19.8 |
| Revenue (MM) ('14E) | 24.0 |
| EV/S ('12) | 1,373.3x |
| EV/S ('13) | 20.8x |
| EV/S ('14) | 17.2x |




## No News Is Good News In CCALD

Last month bluebird announced that it had transplanted the first boy in a Phase II/III trial ('Starbeam") using the company's Lenti-D gene therapy for CCALD. The trial's protocol requires engraftment from the first two individuals before additional patients can be enrolled and features an enrollment target of 12-15 boys. Given the potentially pivotal nature of this study, BLUE will not be providing regular enrollment or other updates. However, the company would likely need to report any significant safety or other adverse findings as they would likely be material. Hence, we view no news as good news and continue to expect enrollment to complete in the 2015 timeframe.

## Beta Thalassemia Trials On Track To Provide Early Data in H2:14

Phase I/II trials on LentiGlobin are still tracking to transplant their first transfusion-dependent beta thalassemia patient this quarter. Investors should expect more regular updates from these studies (one in the U.S., one in France), including possible early data on engraftment rates, Hb production, and transfusion requirements in H2:14. BLUE believes the ability to reduce transfusion rates by >50% in a majority of patients would represent a meaningful advance in this setting. LentiGlobin trials in sickle cell disease will begin enrolling in 2014.

## Expect BLUE's Pipeline And Platform To Expand Over Time

BLUE has achieved critical mass and momentum in the industrialization of lentivirus-based gene therapy. Given the plethora of other gene therapy programs/platforms, many of which reside in academia or smaller companies, we would expect BLUE to expand its reach via a disciplined set of partnerships and acquisitions. However, BLUE's immediate focus is executing on its 3 clinical programs and CELG partnership.

Please see addendum of this report for important disclosures.

## 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, bluebird has over $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.

**Upcoming bluebird bio Milestones**

| Event | Timing |
|---|---|
| Treat first patient in β-thalassemia trials | YE:13 |
| Initiate U.S. trial of LentiGlobin in sickle cell patients | 2014 |
| Initial 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 | Q4:13E | 2013E | Q1:14E | Q2:14E | Q3:13E | Q4:13E | 2014E |
|---|---|---|---|---|---|---|---|---|---|---|
| Lenti-D Revenue | | | | | | | | | | |
| LentiGlobin Revenue | | | | | | | | | | |
| Collaborative and Grant Revenue | 1.1 | 6.3 | 6.4 | 6.0 | 19.8 | 6.0 | 6.0 | 6.0 | 6.0 | 24.0 |
| **Total Revenue** | **1.1** | **6.3** | **6.4** | **6.0** | **19.8** | **6.0** | **6.0** | **6.0** | **6.0** | **24.0** |
| *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 | 8.8 | 30.0 | 8.9 | 9.0 | 9.1 | 9.2 | 36.2 |
| SG&A | 2.3 | 3.3 | 3.8 | 3.8 | 13.2 | 3.8 | 3.8 | 3.9 | 3.9 | 15.4 |
| **Total Expenses** | **7.6** | **10.5** | **12.5** | **12.6** | **43.3** | **12.7** | **12.8** | **13.0** | **13.1** | **51.6** |
| **Operating Income/ Loss** | **(6.5)** | **(4.2)** | **(6.2)** | **(6.6)** | **(23.4)** | **(6.7)** | **(6.8)** | **(7.0)** | **(7.1)** | **(27.6)** |
| Non-Operating Income | (0.1) | (0.4) | 0.0 | (0.1) | (0.5) | (0.2) | (0.2) | (0.2) | (0.2) | (0.8) |
| **Pre-tax Income/ Loss** | **(6.5)** | **(4.6)** | **(6.1)** | **(6.7)** | **(23.9)** | **(6.9)** | **(7.0)** | **(7.2)** | **(7.3)** | **(28.4)** |
| *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)** | **(6.7)** | **(23.9)** | **(6.9)** | **(7.0)** | **(7.2)** | **(7.3)** | **(28.4)** |
| **GAAP EPS** | **($0.41)** | **($2.13)** | **($0.26)** | **($0.28)** | **($1.46)** | **($0.28)** | **($0.28)** | **($0.29)** | **($0.29)** | **($1.15)** |
| Diluted Shares | 16.0 | 2.2 | 23.6 | 23.7 | 16.4 | 24.4 | 24.6 | 24.8 | 24.9 | 24.7 |

Source: Cowen and Company

**bluebird bio Annual P&L Model ($MM)**

| | 2012A | 2013E | 2014E | 2015E | 2016E | 2017E | 2018E |
|---|---|---|---|---|---|---|---|
| Lenti-D Revenue | 0.0 | 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 | 0.0 |
| Collaborative and Grant Revenue | 0.3 | 19.8 | 24.0 | 26.0 | 28.0 | 30.0 | 32.0 |
| **Total Revenue** | **0.3** | **19.8** | **24.0** | **26.0** | **28.0** | **30.0** | **32.0** |
| *Y/Y growth* | | | *0%* | *8%* | *8%* | *7%* | *7%* |
| COGS | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| R&D | 17.2 | 30.0 | 36.2 | 37.0 | 38.0 | 39.0 | 40.0 |
| SG&A | 6.8 | 13.2 | 15.4 | 16.0 | 17.0 | 18.0 | 19.0 |
| **Total Expenses** | **24.1** | **43.3** | **51.6** | **53.0** | **55.0** | **57.0** | **59.0** |
| **Operating Income/ Loss** | **(23.7)** | **(23.4)** | **(27.6)** | **(27.0)** | **(27.0)** | **(27.0)** | **(27.0)** |
| Non-Operating Income | 0.0 | (0.5) | (0.8) | (1.0) | (1.0) | (1.0) | (1.0) |
| **Pre-tax Income/ Loss** | **(23.7)** | **(23.9)** | **(28.4)** | **(28.0)** | **(28.0)** | **(28.0)** | **(28.0)** |
| *Tax rate (%)* | *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 |
| **Net Income (Loss) From Operations** | **(23.7)** | **(23.9)** | **(28.4)** | **(28.0)** | **(28.0)** | **(28.0)** | **(28.0)** |
| **GAAP EPS** | **($1.83)** | **($1.46)** | **($1.15)** | **($1.05)** | **($1.00)** | **($0.95)** | **($0.90)** |
| Diluted Shares | 12.9 | 16.4 | 24.7 | 26.7 | 28.0 | 29.4 | 31.0 |

Source: Cowen and Company

# Valuation Methodology & Investment 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.

## Company Specific Risks

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 Inc |

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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 ALLOCATION

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13

| Rating | Count | Ratings Distribution | Count | IB Services/Past 12 Months |
|---|---|---|---|---|
| Buy (a) | 394 | 58.72% | 54 | 13.71% |
| Hold (b) | 255 | 38.00% | 5 | 1.96% |
| Sell (c) | 22 | 3.28% | 1 | 4.55% |

(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.

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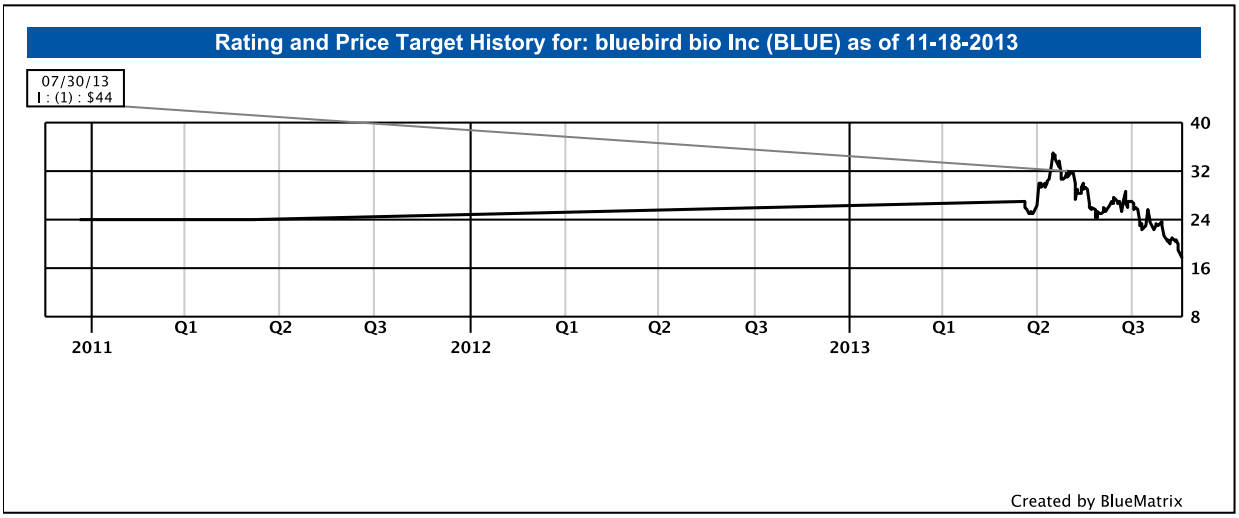


**Legend for Price Chart:**

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