

bluebird bio

I See BLUE, and It Looks Glorious - ALERT

We weren't expecting to quickly bring back additional references to our initiation title for the company that is essentially the polar opposite of "Old School," but Saturday's presentation of initial data from the Phase 1/2 HGB-205 study evaluating BLUE's gene therapy LentiGlobin in beta-thalassemia patients at the European Hematology Association (EHA) Congress in Milan exceeded even the most optimistic expectations and seems more than worthy of the description. We were bullish going into the event (see preview [here](#)) and are no doubt pleased by the early and high-level production of corrected β AT87Q-globin observed (demonstrates that the gene therapy is indeed doing what it's supposed to be doing). However, what's of far greater surprise is that the first two patients treated in this clinical trial not only became *transfusion independent* but did so very rapidly. Based on this rather striking (albeit early) data, we anticipate BLUE shares could be sharply higher on Monday. Reiterate OW.

- **First two patients enrolled in the HGB-205 study show rapid and significant increases in corrected hemoglobin.** At 4.5 mos post-transplant, subject 1 (aka pt #1201) had a total hemoglobin of 10.1 g/dL of which 6.6 g/dL was therapeutic β AT87Q-globin. Subject 2 (#1202) had a total hemoglobin of 11.6 g/dL (4.2 g/dL was β AT87Q-globin) just 2 mos post-transplant. In both of these pts, production of therapeutic hemoglobin looks to be meaningfully greater vs. the best performing pt (#1003) in the prior pilot trial. Recall that production of β AT87Q-globin with that patient appeared ~4 months after initiation of LentiGlobin treatment.
- **Rapid transfusion independence is the shocker of this update.** The first 2 pts had their last blood transfusion just 10 and 12 days, respectively, post transplant and remain transfusion independent. As a reminder, the best pt in the original trial became transfusion independent ~1 yr post transplant (and remains so 72 mos later).
- **Initial performance of new vector is highly encouraging and appears to be translating into clinical benefit.** Consistent with the abstract, BLUE's new and improved vector triggered vector copy numbers (VCNs) of 1.52 and 2.12 in subjects 1 and 2, respectively (~2.5-3.5x higher vs. the best performing pt from the first study who had a VCN of 0.61 after treatment, and pt #1004 who had a VCN of 0.31). Based on the results at EHA, it's looking increasingly likely that the higher VCN is indeed translating into beneficial patient outcomes as hoped.
- **Safety in both the HGB-205 & LG001 studies remains encouraging.** BLUE reported that no drug product related adverse events were seen in either trial (consistent with our expectations and the abstract). Most notably, an integration site analysis after 3 mos in subject 1 showed polyclonal reconstitution.
- **We look forward to additional follow up later this year.** Additional data from the ongoing LentiGlobin studies are expected in late 2014 (we assume at ASH in Dec).
- **BLUE is hosting a conf call on Mon, June 16th at 8am ET.** Dial-in info (844) 825-4408 (US) and (315) 625-3227 (outside the US).

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Overweight

BLUE, BLUE US

Price: \$26.09

13 June 2014

Biotechnology

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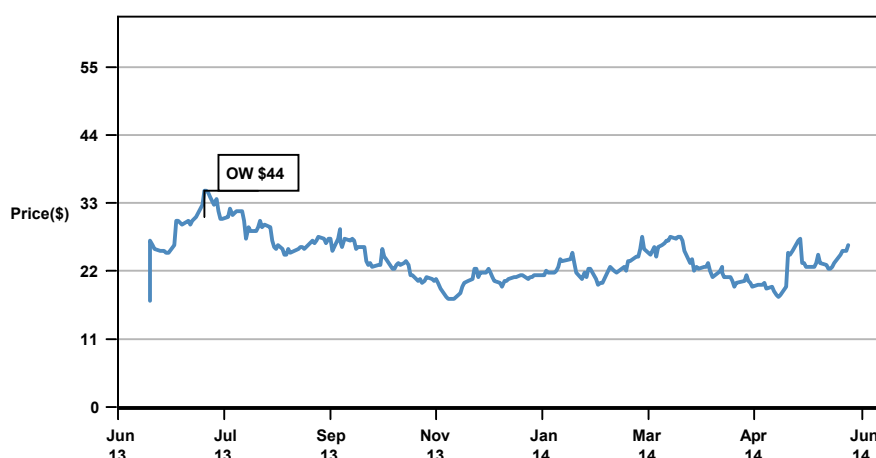
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bluebird bio (BLUE, BLUE US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	30.65	44.00

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Jul 15, 2013.

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