bluebird bio

EHA Abstract Has Promising Potential Read-Through to Clinical Benefit; Updated Data at the Conf in June - ALERT

Overweight

BLUE, BLUE US
Price: $27.21

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Biotechnology
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Today, an abstract highlighting preliminary data to be presented at the European Hematology Association (EHA) Congress to be held in Milan, Italy (June 13-16), from the ongoing Phase 1/2 HGB-205 Study of LentiGlobin (gene therapy) for beta-thalassemia major was posted online. While the abstract does not contain any clinical outcomes data from the HGB-205 study (which is our best guess as to why shares were weak today), we are encouraged that it does show that the vector copy number (VCN) has improved dramatically (by 2.5-3.5x) using the company's new optimized vector (BB305) vs. the best-performing patient (who achieved transfusion independence for 5+ years) using the old vector (HPV569) in the prior study (the LG001 trial). It still remains to be seen if the higher VCN, as well as the faster time-to-neutrophil engraftment, in the HBG-205 trial translates over into clinical benefit; we will get our first look at data on the transplant outcomes in 2 patients with up to 6 months of follow-up at the EHA meeting itself. Overall, we continue to view BLUE – with its gene therapy platform – as a potentially transformative and disruptive company that appears to be more than just a "big idea" and recommend owning shares in front of upcoming clinical trial updates. Reiterate OW.

- **Abstract data show impressive vector copy numbers with the new vector.** In the abstract, patients #1201 and #1202 (from the HGB-205 study) show VCNs of 1.52 and 2.12, respectively. For context, this is 2.5-3.5x higher vs. the best-performing patient (patient #1003 who has remained transfusion independent for 5 years and counting, with a VCN of 0.61 after treatment) using the old vector (HPV569) in the prior study (the LG001 trial). For additional context, patient #1004 from the prior LG001 trial showed a VCN of 0.31 (with the old HPV569 vector), and while she remains transfusion dependent, βA-T87Q-globin accounts for ~5% of total hemoglobin 2 years post-treatment. In addition, the new vector also showed a faster time-to-neutrophil engraftment than the prior vector (14 days vs. 20-29 days for patients #1003 and #1004). The question now is whether this higher VCN and faster time-to-neutrophil engraftment with the new BB305 vector will translate into clinical benefit in patients in the HGB-205 trial.

- **Upcoming events: clinical update next month at EHA.** Clinical data from the ongoing Phase 1/2 HGB-205 Study of LentiGlobin for the treatment of beta-thalassemia major will be presented at the European Hematology Association (EHA) Congress to be held in Milan, Italy, over June 13-16. BLUE also expects to present additional Phase 1/2 data for LentiGlobin in beta-thalassemia (from the HGB-205 and Northstar studies) in late 2014. Finally, BLUE now has an active IND for LentiGlobin in the US for sickle cell disease (SCD); the US trial will be called HGB-206, and the company expects to transplant the first SCD patient with LentiGlobin in 2014 (in the HGB-205 or HGB-206 studies).

See page 2 for analyst certification and important disclosures.

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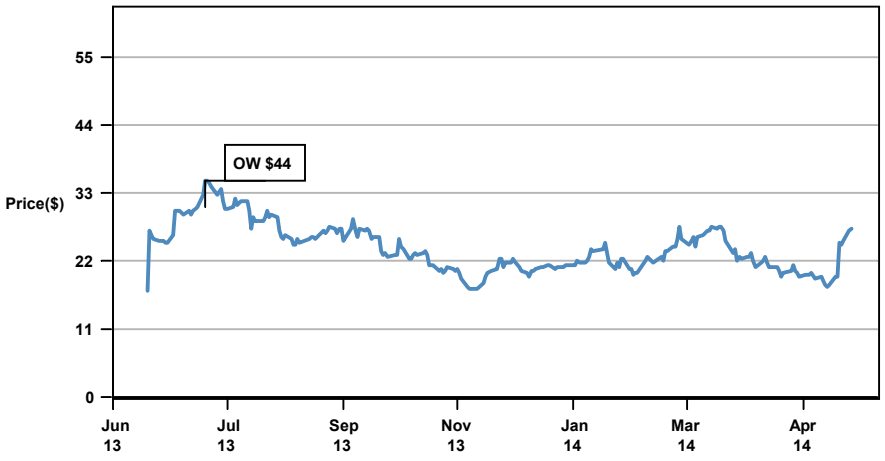
bluebird bio (BLUE, BLUE US) Price Chart



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.
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