June 15, 2014

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bluebird bio (BLUE - OUTPERFORM): LentiGlobin Produces Early Transfusion Independence in First Two Beta-Thal Patients in Phase I/II Study, Reiterate OUTPERFORM

Price: $26.09 **12-Month Price Target: $40**

- **Additional data from the ongoing Phase I/II (HGB-205) study shows that LentiGlobin was effective in helping beta-thal patients achieve transfusion-independence.** Data presented at the European Hematology Association (EHA) Congress on June 14 shows that two transfusion-dependent patients with beta-thalassemia became transfusion free (3.5 and 6.5 months) within two weeks (10 and 12 days) following treatment with the improved BB305-LentiGlobin vector.
- **The clinical benefit observed in HBG-205 is more rapid than that seen with the prior-generation LentiGlobin vector.** Follow-up data from the Phase I/II LG001 study shows that one patient (Subject 3) treated with the HPV569-LentiGlobin vector has remained transfusion free for six years, although transfusion independence was not achieved until one year post-treatment. No treatment-related AEs have been observed in either LentiGlobin study.
- **The results in HGB-205 are a reflection of the improved transduction efficiency of the new vector.** The two patients in the HBG-205 study were producing 4.2 and 6.6 g/dL of T87Q-corrected globin (36% and 65% of total hemoglobin produced, respectively) at 2 and 4.5 months post-treatment. This expression is higher and occurred earlier than with the prior-gen vector, with Subject 3 producing a peak of 2.7 g/dL of corrected globin (about 30% of total hemoglobin) at 1.5 years following treatment. The EHA abstract released in late May also shows the new vector produced a higher (1.52-2.12) vector copy number (VCN) compared to the prior-gen vector (0.61 VCN in Subject 3). Given the superior VCN and hemoglobin production observed with the improved LentiGlobin vector, patients in the HGB-205 study could have an even more consistent benefit than that seen with the earlier generation vector (and potentially a life-long cure).
- **Management will host a conference call Monday morning to discuss the results, which could provide insights into whether this initial data could result in a more rapid approval for LentiGlobin in beta-thal.** The call will take place at 5 am Pacific and can be accessed by dialing 844-825-4408 (315-625-3227 if outside U.S.) or through a webcast on the Events page at www.bluebird-bio.com. The French HGB-205 study and the U.S. Phase I/II Northstar study continues to enroll beta-thal patients, with additional data from both studies expected late this year.
- **Reiterate OUTPERFORM and our $40 price target.** Our price target is derived from applying an 8X multiple to estimated 2020 revenues for Lenti-D and LentiGlobin products, discounted 35% annually.

Risks to the achievement of our price target include failure to gain approval for products in development, failure to achieve sales estimates for any marketed product and failure to achieve earnings estimates.

Analyst Certification
I, David M. Nierengarten, Ph.D., Dilip Joseph, Liana Moussatos, Ph.D., certify that the views expressed in this report accurately reflect my personal opinion and that I have not and will not, directly or indirectly, receive compensation or other payments in connection with my specific recommendations or views contained in this report.

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Company	Disclosure
bluebird bio	1,3,4,5

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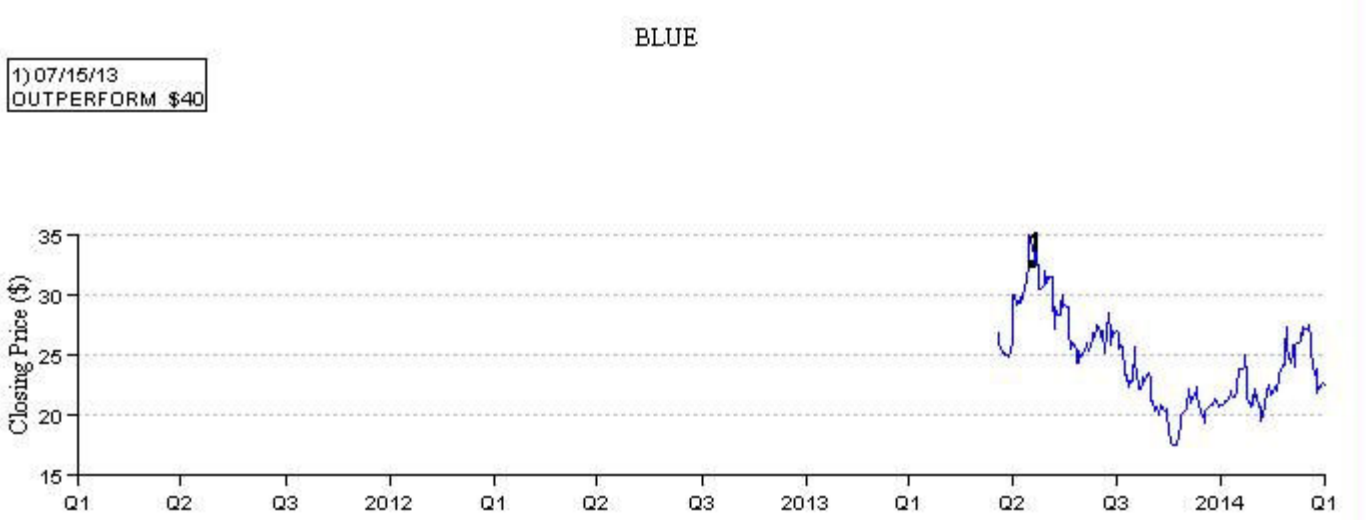

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