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bluebird bio (BLUE - OUTPERFORM): Preliminary Phase I/II Data in Beta-Thal Suggests Potential for Improved Clinical Outcomes with LentiGlobin, Reiterate OUTPERFORM

Price: \$22.23 12-Month Price Target: \$40

- Encouraging initial results from an ongoing French Phase I/II (HGB-205) study of LentiGlobin in beta-thalassemia points to improved transduction efficiency for the new vector. The study results will be presented at the European Hematology Association Congress in Milan (June 13-16). An abstract released this morning included data from two patients who underwent treatment in the HGB-205 study and follow-up data from two patients treated with the earlier generation LentiGlobin vector (HPV569) in the Phase I/II LG001 study. The new vector (BB305) used in HGB-205 was shown to produce a faster time-to-neutrophil engraftment (14 vs. 20-29 days) and a higher (1.52-2.12 vs. 0.31-0.61) vector copy number (VCN) compared to the prior-gen vector.
- Although limited, the data hints at the potential for improved clinical outcomes with the new vector. Follow-up data from the LG001 study shows that one patient remains transfusion-independent at five years, with a VCN of 0.23 and high level of expression for the T87Q-corrected globin (~30% of total hemoglobin). Considering that the one patient in the HGB-205 study with complete data had a superior VCN and time-to-engraftment, we would expect the new vector to result in an equal-or-better clinical outcome as the study progresses. We expect additional data from the HGB-205 study to be presented in late 2014 (likely at ASH) to provide further insight into the transduction efficiency and clinical outcomes of the new LentiGlobin vector.
- We believe the selloff today is more a reflection of the limited quantity of data made available today, rather than the quality of the data.
- **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.

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Company	Disclosure
bluebird bio	1,3,4,5

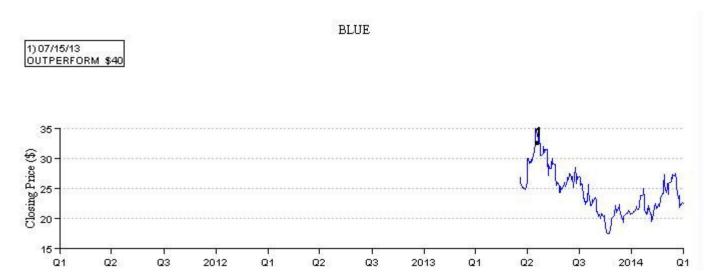
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