

December 2, 2013

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## bluebird bio (BLUE - OUTPERFORM): First Patient Enrolled in Beta-thal Study, Reiterate OUTPERFORM

Price: \$20.60

12-Month Price Target: \$40

- Bluebird bio announced that the first patient with beta-thalassemia has been treated with LentiGlobin—the company's gene therapy treatment—in its Phase I/II HGB-205 study of LentiGlobin treatment of patients with beta-thalassemia and sickle cell disease (SCD). The French trial is designed to be an open-label study in up to seven patients with beta-thal or severe SCD.
- We expect initial data in late 2014, with efficacy in beta-thal patients measured by transfusion requirements post-transplant and number of hospitalization days (for SCD patients efficacy will be measured by number of vaso-occlusive crises or acute chest syndrome events). A separate Phase I/II study in the US for LentiGlobin in beta-thal is currently recruiting patients.
- Recall that three patients with beta-thal had been treated with a prior generation gene therapy vector. One patient became transfusion-independent about one year post-transplant. We expect at least similar if not better efficacy in the HGB-205 study evaluating the improved LentiGlobin vector.
- We estimate LentiGlobin launching in 2019 with rapid adoption leading to \$1.24B in estimated revenues in 2020, our valuation year. Our pricing per treatment of \$450K in the US and Europe and \$150K in Asia is reasonable based on the estimated lifetime cost of care for beta-thal/SCD patients and the curative potential of LentiGlobin.
- Earlier this quarter, BLUE transplanted the first patient in the pivotal Starbeam (ALD-102) study for the company's lead gene therapy candidate Lenti-D for childhood cerebral adrenoleukodystrophy (CCALD).
- Reiterate OUTPERFORM rating and \$40 price target. Our price target of \$40 is derived from applying an 8X multiple to estimated 2020 revenues of \$1.36B for LENTI-D and LENTIGLOBIN products, discounted 35% annually. We do not include CAR-T (chimeric antigen receptor T-cells, partnered with Celgene (CELG, not covered) in our valuation.
- 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	1,3,5

### Research Disclosure Legend

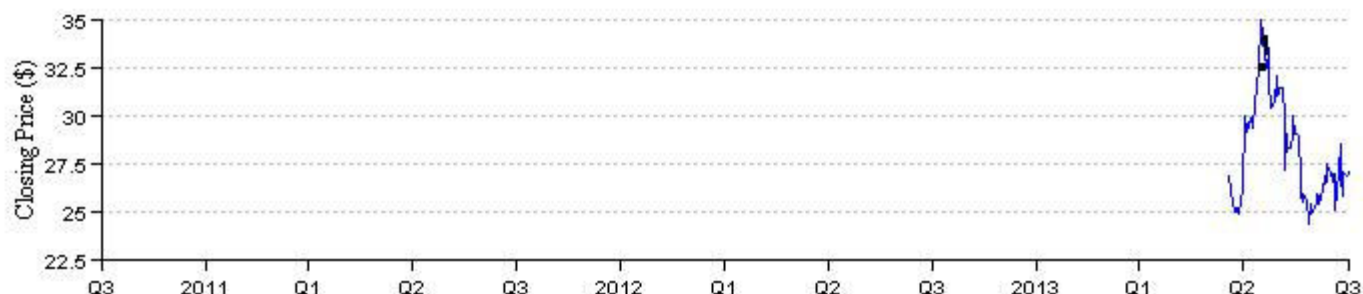
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BLUE

1) 07/15/13  
OUTPERFORM \$40



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