bluebird bio, Inc. (BLUE) — Overweight

LentiGlobin Data at EHA Shines Bright

CONCLUSION

Over the weekend at the EHA conference, BLUE reported data from two patients with beta-thalassemia treated with its LentiGlobin gene therapy product. Previously reported vector copy number (avg number of lentivirus vectors/cell) in these patients had left us optimistic these results would be good, and the clinical benefit in hemoglobin was substantially more impressive than we had expected. Our first impression is that these results not only solidify LentiGlobin prospects in beta-thal but also positions it attractively for the sickle cell indication which is also being pursued; so far we have only assumed modest contribution for the BT indication in our BLUE valuation. We'll look to get additional details during a conference call BLUE is hosting tomorrow morning at 8AM EST to discuss results. We reiterate our Overweight rating and expect shares to react favorably to this update.

- **That is cool stuff.** Two beta-thal patients were treated with BLUE's optimized lentivirus vector; both patients rapidly became transfusion independent. One had a total hemoglobin (Hb) of 10.1g/dL of which an impressive 6.6g/dL was from the corrected gene replacement while the other had a total Hb of 11.6g/dL of which 4.2g/dL was from the corrected gene replacement. The ability to create such a high degree of normalized Hb from gene therapy represents an impressive proof of concept for this platform and a potentially major step forward for this important unmet medical need.

- **Helps validate sickle too.** Both beta-thal and sickle cell anemia are genetic diseases affecting hemoglobin. Sickle cell crises are often treated or prophylaxed with blood transfusions, so the ability to correct so much Hb in the BT setting bodes well for an impactful correction not just of sickle cell patients dependent on transfusions to maintain Hb levels but also to prevent potentially catastrophic sickle crises.

- **Sets a high bar for the competition.** SGMO/BIIB plan to file an IND for their own gene therapy program for beta-thal by YE. Their approach differs in that instead of adding a normal globulin gene, Zn fingers are being used to knock out the Bcl11a promoter which is responsible for the switch from fetal hemoglobin to adult hemoglobin. Increasing fetal hemoglobin levels to replace the defective adult hemoglobin gene may represent a genetic engineering approach to treat these disorders without a genetic integration requirement. Whether one of these approaches will prove to be a 'winning' strategy remains to be seen; in the absence of a winner the market should be large enough to accommodate two sizable gene therapy products.

- **Lovin' this field.** We've spent a fair amount of time assessing the evolving gene therapy platform; yet another validating datapoint should be considered a positive for the field overall as well as BLUE.

COMPANY DESCRIPTION

BLUE is one of the pioneers in gene therapy.

PRICE: US$26.09
TARGET: US$34.00
DCF through 2022 using 11% disc rate, 8% terminal growth & 36x terminal multiple.

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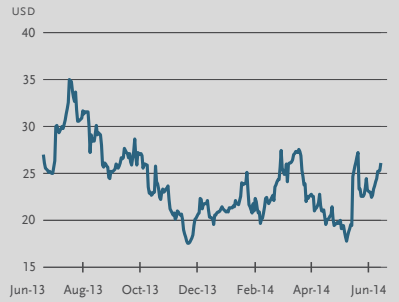
RISKS TO ACHIEVEMENT OF PRICE TARGET

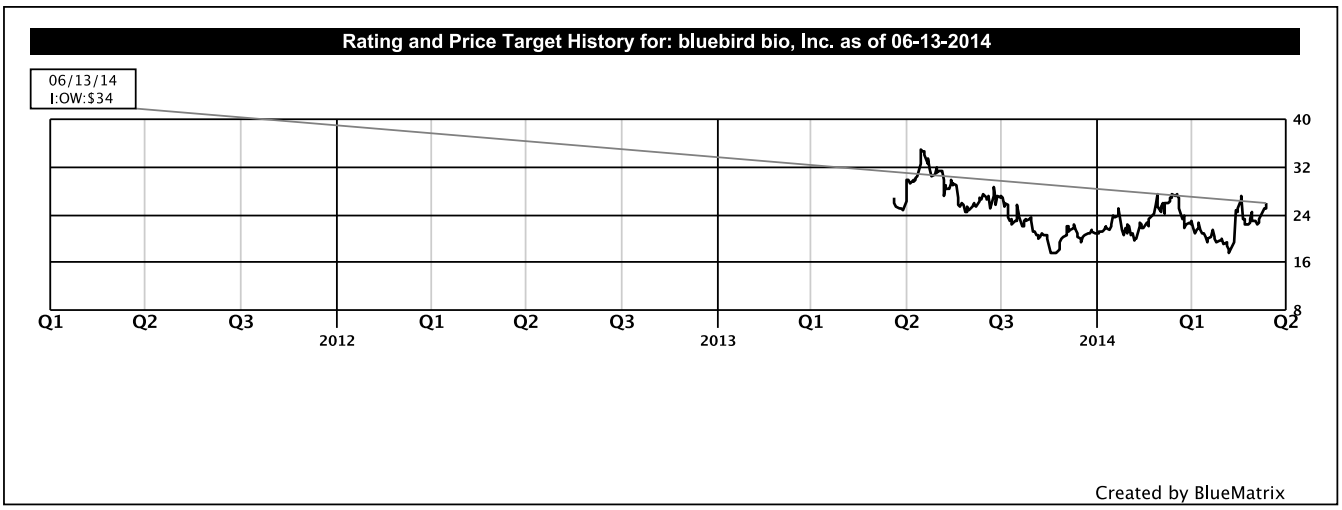
BLUE or the gene therapy field may face development or regulatory setbacks.

Price Performance - 1 Year



Source: Bloomberg

IMPORTANT RESEARCH DISCLOSURES



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R: Resuming Coverage
T: Transferring Coverage
D: Discontinuing Coverage
S: Suspending Coverage
OW: Overweight
N: Neutral
UW: Underweight
NA: Not Available
UR: Under Review

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Rating	Count	Percent	Count	Percent
BUY [OW]	355	61.95	87	24.51
HOLD [N]	203	35.43	21	10.34
SELL [UW]	15	2.62	0	0.00

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