

bluebird bio, Inc. (BLUE)

Overweight

Acquisition Of Pregen Strengthens BLUE's Position

CONCLUSION

This afternoon, BLUE announced its acquisition of Pregen, a private, Seattle-based gene editing company. We believe this acquisition strengthens BLUE's IP position and complements BLUE's current pipeline as well as strategy to pursue synergistic platforms, which will allow the company to remain competitive in the constantly evolving gene therapy space. We reiterate our OW rating and \$58 PT.

- Details of the acquisition.** BLUE issued 408,667 shares of its common stock and agreed to pay \$4.9M of Pregen's current liabilities. Pregen will also be entitled to an additional \$135M if certain pre-clinical, clinical, and commercial milestones are met. In return, BLUE has gained access to Pregen's extensive IP portfolio and "know-how" for gene editing and cell signaling technologies, which potentially have a broad range of therapeutic applications in hemoglobinopathies, CAR-T, cancer immunotherapy, and beyond. According to BLUE management, this acquisition will help differentiate the company's CAR-T program (partnered with CELG), and the advantages offered by Pregen's gene editing platform should become apparent over the next year. Collectis current is advancing its own CAR-T program with the added benefit of its gene editing platform which we see as a distinct advantage, so BLUE being competitive on this front is important.
- Understanding Pregen's platform.** Pregen's technology includes homing endonucleases (HEs) and megaendonuclease/TALEN (MegaTALs). HEs are naturally occurring proteins that bind to DNA in a sequence specific manner and cleave the targeted sites. HEs can target DNA sequences greater than 20 base pairs, and are designed to promote the editing or replacement of a defective gene through the body's natural cellular DNA repair mechanisms. MegaTALs are fusion proteins that combine HEs with the targeting domain of transcription activator-like effector nucleases (TALENs). According to BLUE management, the work done by Pregen has resulted in a gene editing technology with greater DNA sequence specificity and lower off-target activity. The benefit of this technology should be most apparent (at least initially) in improving the functionality of T-cells for CAR-T therapy. Beyond CAR-T, HEs and MegaTALs can be expanded into future targets and indications as well as expansion into in vivo administration with viral and nonviral vectors.
- What's next for BLUE.** During EHA in mid-June, BLUE presented data in 2 beta-thalassemia (BT) patients, which beat expectations. The company will initiate its US Phase 1/2 (HGB-206) study in SCD patients during 2H14. Look for additional BT and SCD data YE14, as well as potential updates on the BLUE/CELG CAR-T program during 2015.

COMPANY DESCRIPTION

BLUE is one of the pioneers in gene therapy.

PRICE: US\$38.57

TARGET: US\$58.00

DCF through 2022 using 10.5% disc rate, 8% terminal growth & 36x terminal multiple.

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Related Companies:

BLUE

Share Price:

38.57

RISKS TO ACHIEVEMENT OF PRICE TARGET

Failure to achieve clinical, regulatory and development targets. Insertional mutagenesis.

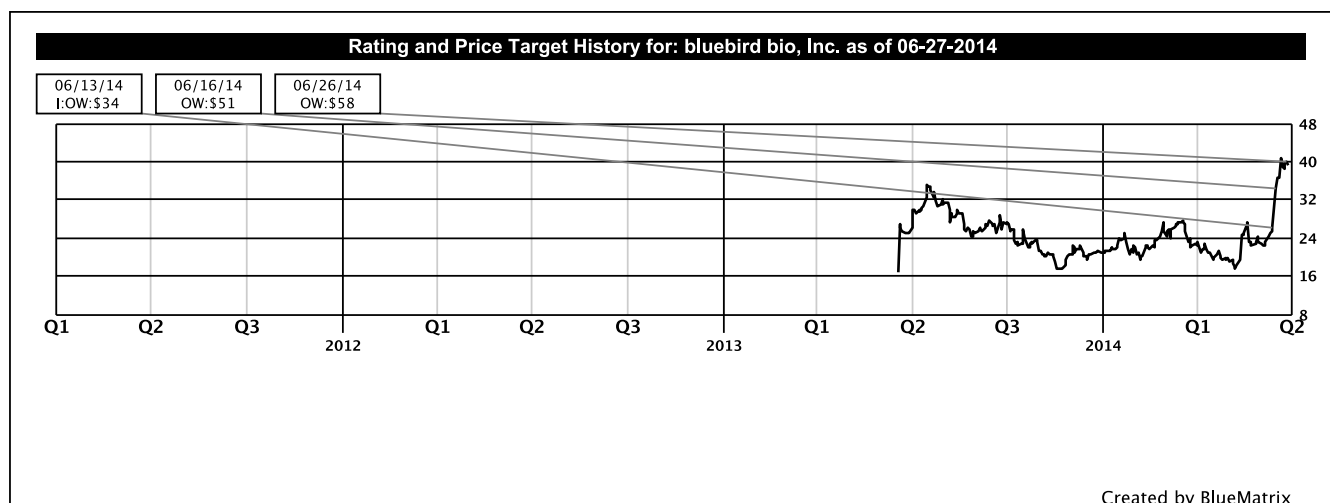
Note: price as of the close June 30, 2014

Price Performance - 1 Year



Source: Bloomberg

IMPORTANT RESEARCH DISCLOSURES



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

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BUY [OW]	356	61.91	87	24.44
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Analyst Certification — Joshua E. Schimmer, MD, Sr Research Analyst — Kristina N. Cibor, Research Analyst

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