

bluebird bio

Pregenen Acquisition Adds to BLUE's Disruptive Tech Platform - ALERT

Today after the close, BLUE announced the acquisition of Precision Genome Engineering, Inc. (aka Pregenen), a private biotech company that specializes in the development of gene editing and cell signaling technologies. We see this deal as an incremental positive given the relatively low cost for a complementary technology (gene editing), which BLUE believes is a natural progression in the evolution of gene therapy. While no details were given on how/when the newly acquired technology might show up in a BLUE product, the company did indicate there is potential for it to have additive therapeutic effect in both existing programs and new indications. Overall, we continue to view BLUE—with its gene therapy platform and now newly acquired gene editing capabilities—as a potentially transformative and disruptive company, and thus we are reiterating our Overweight rating.

- **The acquisition provides BLUE access to Pregenen's gene editing technology, with broad IP and proprietary knowhow.** While BLUE didn't elaborate on its plans for this technology, it did indicate that the gene editing platform has the potential to benefit both existing programs (hemoglobinopathies, CAR-T) as well as broad potential new applications. Specifically to CAR-T, BLUE plans to work with CELG/Baylor to determine how to incorporate the gene editing tech into the existing programs.
- **Pregenen's HE and megaTAL approach is differentiated in multiple ways from competitive genome editing technologies.** BLUE noted that in all of its diligence, the HE/MegaTAL technology piqued its interest early, and with a focus on Pregenen specifically given its IP and foundational team. HEs are the only naturally occurring proteins that bind in a DNA specific fashion and cleave target sites. While these proteins do occur in nature, Pregenen's IP comes from the database/proprietary knowhow that give the ability to generate sequence specific variants (which are all patentably distinct). MegaTALs are fusion proteins of HEs and TALENs, which provide for greater specificity/lower off-target activity. Further, HE/MegaTALs are compact in size, allowing for delivery of multiple proteins (via viral or non-viral delivery methods).
- **Pregenen will become a wholly owned subsidiary of BLUE, for relatively modest upfront investment.** Pregenen consists of six employees and lab space sufficient to develop the nuclease platform. BLUE noted that it plans to build out the footprint in Seattle using Pregenen as a foundation. Upfront, BLUE issued Pregenen stakeholders ~409K shares and assumed ~\$4.9M in current liabilities. The former stakeholders are also eligible to receive up to an additional \$15M in cash upon certain preclinical milestones, \$20.1M on certain clinical milestones, and \$99.9M upon the achievement of certain commercial milestones.

Overweight

BLUE, BLUE US

Price: \$38.57

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Biotechnology

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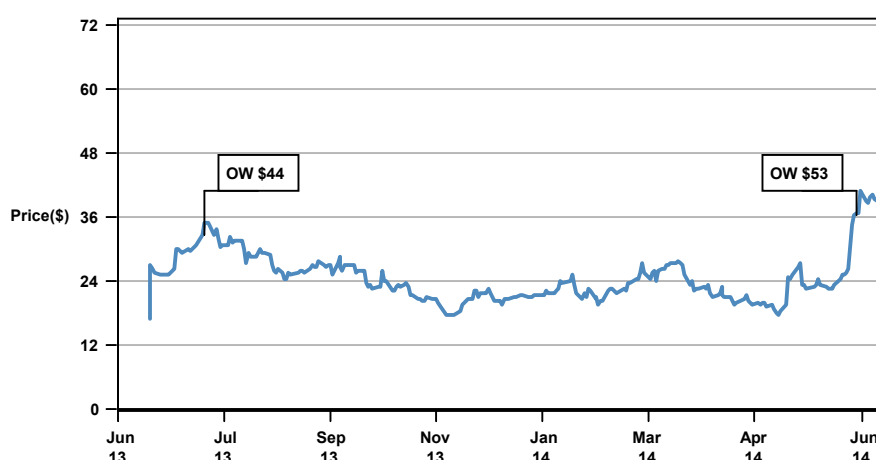
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bluebird bio (BLUE, BLUE US) Price Chart



| Date | Rating | Share Price (\$) | Price Target (\$) |
|-----------|--------|------------------|-------------------|
| 15-Jul-13 | OW | 32.50 | 44.00 |
| 17-Jun-14 | OW | 36.47 | 53.00 |

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Jul 15, 2013.

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