

PTC Therapeutics

Key Takeaways From Our CEO Call - ALERT

This morning, as part of our 2014 CEO/CFO Conference Call Series, we hosted PTC Therapeutic CEO Stuart Peltz. Focus of the call was largely on ataluren in Duchene muscular dystrophy (DMD). Indeed, enrollment in the ongoing phase 3 ACT study remains on track to complete in mid-2014 with data in mid-2015. This study has a high probability of success given careful patient enrollment and sufficient powering, in our view. Additionally, start of a confirmatory phase 3 trial for ataluren in cystic fibrosis (CF) remains on track for 1H14 and the SMA program has recently advanced into the clinic. Given the continued progress and our nmDMD expectations, we reiterate our Overweight rating. **We will have a transcript of the call early next week and a replay of the call is available through 3/15 at 888-567-0440 (US); +1-203-369-3442 (OUS); Passcode: 2814.**

- **Ataluren in nmDMD:** Recall, enrollment in the phase 3 ACT trial for ataluren in nmDMD is ongoing. PTC reiterated expectations for enrollment to compete in mid 2014 with data in mid-2015. PTC highlighted the importance of natural history data (confirmed by two independent groups) in designing the ACT study. Importantly, entry criteria were used to enrich for patients in the decline phase of the disease that maximizes the probability of success, in our view.
- **Conditional approval in Europe:** A final decision on conditional approval in Europe for ataluren in nmDMD is expected in 2Q14. Recall, the CHMP adopted a negative opinion in January and, as such, approval is unlikely. However, PTC noted that going through the process helped to educate European regulators, which should set a good foundation for future regulatory discussions in 2H15 when data from the ACT trial are available.
- **Ataluren in nmCF:** A confirmatory phase 3 trial for ataluren in CF remains on track to begin in 1H14 with data expected in mid-2016. PTC noted the key endpoints will include FEV1 (primary) and pulmonary exacerbations. Importantly, based on a finding in a prior study use of tobramycin will be restricted.
- **Other ataluren indication:** PTC highlighted the potential of ataluren in other indications beyond nmDMD and nmCF. Of note, there are >20 publications demonstrating ataluren is active in multiple indications. PTC is currently in the process of prioritizing these additional indications to advance into development either with ataluren or the next generation agent.
- **SMA:** Recall, the SMA program is partnered with Roche. Phase 1 clinical development began earlier this year with a single ascending dose study that will likely be followed by a multiple ascending dose study later this year. PTC highlighted preclinical data that demonstrated potent activity for this agent.
- **Reiterate Overweight rating.**

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Overweight

PTCT, PTCT US

Price: \$31.98

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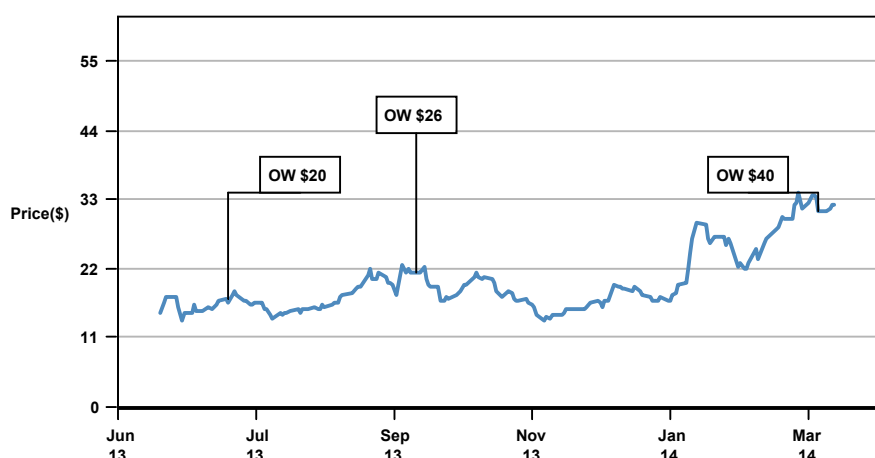
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Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Jul 15, 2013.

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