

PTC Therapeutics

Breakout Session and Presentation Highlights -
ALERT

This afternoon PTC CEO Dr. Stuart Peltz presented at the J.P. Morgan Healthcare Conference. Dr. Peltz provided a comprehensive overview of ataluren's biology and clinical development, and outlined key milestones in 2014. These include completion of enrollment for ataluren in DMD (mid-2014) and initiation of a phase 3 study in CF in 1H14. Additionally, the company disclosed that a CHMP decision is now expected on ataluren conditional approval by the end of Jan 2014 (updated from 1Q14), though expectations of a positive decision remain low (in line with previous comments). Similarly, a phase 1 study for the SMA program is expected to start in 2014. Based on the significant opportunity for ataluren in nmDMD, we reiterate our Overweight rating.

- **Highlights from the breakout sessions. Phase 3 DMD trial design** – PTC discussed multiple aspects of the phase 3 design, including patient enrichment, which should improve the probability of success relative to the phase 2 study. **CHMP conditional approval** – Though the company has stated that its base case assumes EU approval based on data from the phase 3 study, it noted that it has already undertaken planning to launch should conditional approval be granted later this month. **Development in SMA** – The company underscored the potential application of the alternative splicing platform to indications beyond SMA, including DMD (exon 51) and oncology.
- **Development in DMD.** PTC reaffirmed timelines for the phase 3 study in nmDMD (complete enrollment in mid-2014, data in 2H15). The company highlighted insights from the phase 2 study, and changes in the phase 3 study, which should increase the probability of success. Recall, the phase 2 study nearly hit statistical significance in a less selective patient population. PTC noted that in a population similar to the phase 3 enrollment criteria, the drug achieved a 50-meter improvement in 6MWT relative to placebo.
- **CHMP conditional approval.** The most notable event in 2014 is a decision by the CHMP on conditional approval of ataluren in nmDMD in 1Q14. We view this as a low probability event; approval would, however, drive meaningful upside with an EU launch that could be two years ahead of expectations.
- **Ataluren development in CF.** PTC reaffirmed timelines for the phase 3 study with ataluren in CF (initiate 1H14; unchanged). Enrollment is expected to close in 2015, and read-out in 2016.
- **Spinal Muscular Atrophy.** PTC noted that its drug for SMA (partnered with Roche) is anticipated to enter the clinic in 2014. The company also noted that trial could serve as validation of the platform, spurring development in other therapeutic settings.
- **Reiterate Overweight rating.**

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Overweight

PTCT, PTCT US

Price: \$19.87

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Biotechnology

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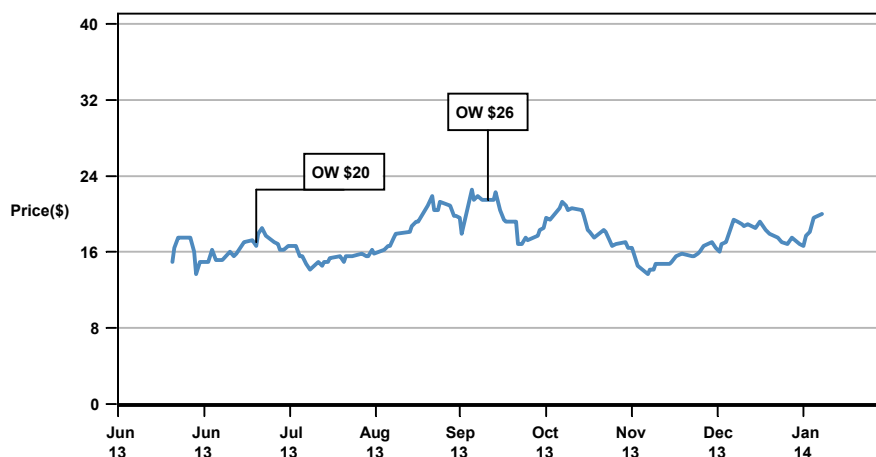
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PTC Therapeutics (PTCT, PTCT US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	16.94	20.00
27-Sep-13	OW	21.37	26.00

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

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