

## PTC Therapeutics

## Key Takeaways from Analyst Day - ALERT

We attended PTC's R&D Day today and wanted to pass along a few key takeaways from the meeting. Overall, we came away very impressed with the depth and breadth of the science and technology platform at PTC. At its core, PTC is an orphan disease focused company with ataluren in DMD and CF and significant potential in other orphan indications. However, there are other programs nearing phase 1 trials such as SMA, oncology (stem cell focus) and infectious diseases (gonorrhea) that look to potentially be very differentiated assets. Of note, the lead program, Ataluren in nmDMD, was only reviewed briefly; however, the company continues to highlight patients enrolling in the phase 3 trial are in the declining phase of the disease. We are reiterating our Overweight rating.

- **Ataluren in nmDMD:** The company highlighted 6MWD is a validated endpoint and that Natural History studies validate this. Recall, the phase 3 trial is powered to show a 30 meter difference relative to placebo. Importantly, PTC reiterated the changes in the phase 3 inclusion criteria from phase 2b (a more heterogeneous population) would have resulted in a ~50m 6MWD benefit (known data). We note phase 3 enrollment is expected to complete mid-2014, with data in mid-2015 (ataluren only drug in development for nmDMD).
- **SMN2 for spinal muscular atrophy (SMA):** Recall, this program is partnered with Roche/SMA Foundation and an orally-available development candidate was identified in August 2013; PTC highlighted Roche is really pushing development. PTC's pre-clinical work that shows increases in SMN protein in in-vitro SMA patient cells, as well as encouraging animal data (good oral bioavailability, dose dependent increases in SMN protein in brain/PBMCs, prolonged survival, and weight gain). We expect the program will enter the clinic in 2014 with likely some proof-of-concept efficacy parameters in the study.
- **Ataluren in nmCF:** There was not much reviewed related to ataluren in CF. PTC reviewed preclinical data that showed ataluren increases CFTR expression in intestine and CFTR chloride channel activity, which was also validated in the phase 2a trial. Recall, post a phase 2/3 failure of ataluren in CF (inhaled antibiotics which interfered with drug activity), a phase 3 study is expected to begin in 1H14.
- **Pipeline – Other:** Ataluren – PTC highlighted potential in other disease models, including limb girdle dystrophy, Hurler syndrome, and Usher syndrome, as well as other publications in other various diseases (supported by pre-clinical data). PTC596 (BMI1 program) – PTC596 is being developed in oncology for a refractory stem cell population, and PTC reviewed scientific rationale for targeting BMI1, as well as pre-clinical data. The lead indication will be glioblastoma (temozolomide-resistant population) and IND-enabling safety studies are ongoing. Antibacterial program – A drug candidate is expected to be identified in 1H14 (PTC847 could be the lead drug candidate in gonorrhea, which has impressive pre-clinical data).
- **Reiterate Overweight rating.** *Roche is covered by JPM analyst Richard Vossler*

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## Overweight

PTCT, PTCT US

Price: \$20.48

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## Biotechnology

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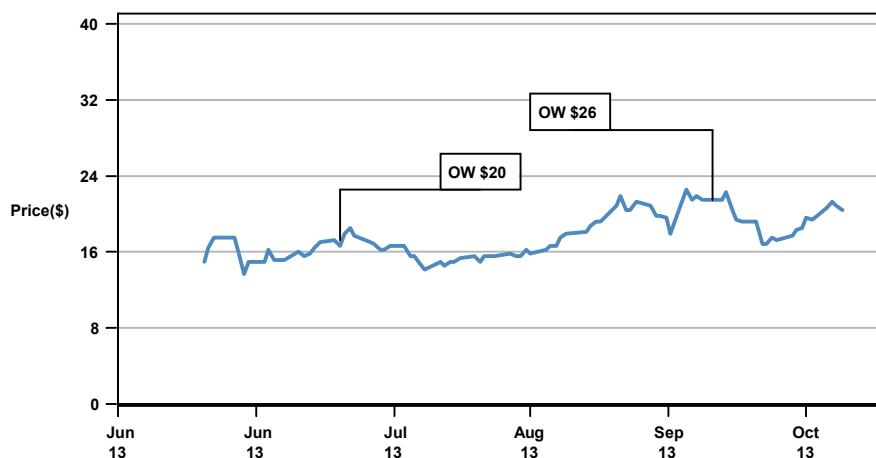
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PTC Therapeutics (PTCT, PTCT US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	17.17	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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