

## PTC Therapeutics

### As Expected, CHMP Does Not Grant Conditional Approval for Ataluren in nmDMD - ALERT

This morning, PTC announced that the CHMP has adopted a negative opinion on ataluren in Duchenne muscular dystrophy (nmDMD). Recall, an MAA for ataluren was filed with the EMA based on phase 2a data, and the 120-day questions (March 2013) raised several major objections. As such, conditional approval was very unlikely to begin with, and no one on the Street had modeled a launch prior to 2016. Importantly, this has no impact on the ongoing phase 3 confirmatory study of ataluren in nmDMD that should complete enrollment in mid-2014 with data in mid-2015. PTC noted that the CHMP raised concerns that conditional approval could impact enrollment in the ongoing confirmatory phase 3 study; the company intends to request a re-examination of the decision with a response from the CHMP expected in 2Q14, but again, approval is not assumed near term in Street models. We continue to believe phase 3 data will be needed for approval, and we recommend buying PTCT shares on weakness today.

- Phase 3 ataluren trial in nmDMD on track to complete enrollment in mid-2014:** In a phase 2b trial, ataluren resulted in a 31-meter improvement in 6MWD compared to placebo ( $p=0.0561$ ) in nmDMD (50m in subgroup matching the phase 3 enrollment criteria;  $p=0.0096$ ). A phase 3 trial is ongoing and expected to complete enrollment in mid-2014, with data in mid-2015. We continue to believe insights gained in phase 2 have led to a well designed and powered global phase 3 trial with a maximized probability of success in nmDMD as well in nm Cystic Fibrosis (phase 3 starting in mid 2014).
- Reiterate Overweight rating.**

## Overweight

PTCT, PTCT US

Price: \$26.22

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### US Biotechnology

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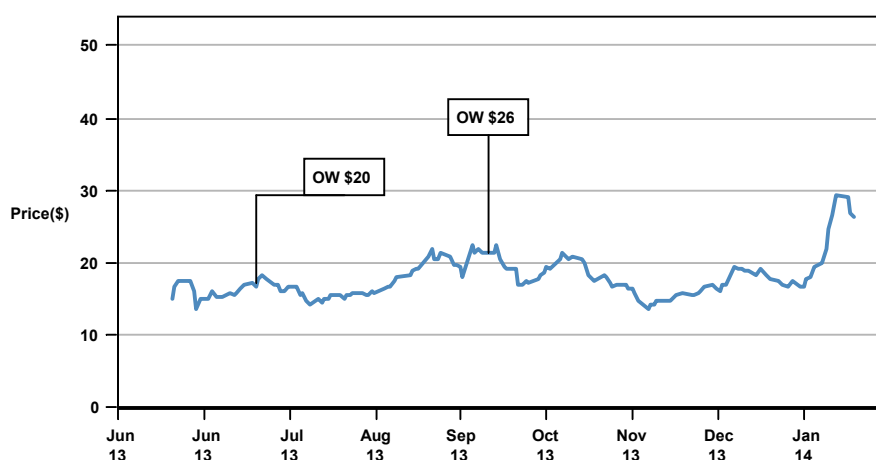
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PTC Therapeutics (PTCT, PTCT US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	17.17	20.00
27-Sep-13	OW	21.45	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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