

PTC Therapeutics

Ataluren Receives Positive CHMP Opinion; Major Upside Surprise - ALERT

This morning, PTC announced that the CHMP has issued a positive opinion for conditional approval of Translarna (ataluren) in nonsense mutation Duchenne Muscular Dystrophy (DMD). This is a major upside surprise, as we and everyone on the Street expected a formal phase 3 would be needed for approval, given a prior negative opinion. We now expect a formal EU launch in early 2015 rather than year-end 2016. Of note, for reference, we estimate ataluren revenues in the EU could easily exceed \$20M in 2015 and \$100M in 2016 (see details below). On the conference call this morning, our focus will be on ataluren EU market access, pricing / reimbursement dynamics, and impact to phase 3 trial. Reiterate Overweight rating.

- **Potential EP impact:** Our model currently assumes an EU launch of ataluren in nmDMD in late 2016. With the positive CHMP opinion, we believe a launch could now occur in early 2015. Bringing our EU launch forward would increase our EPS estimate by \$0.50-2.00 in the outer years.
- **Phase 3 ataluren trial in nmDMD on track to complete enrollment in mid-2014:** In the 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; $p=0.0096$). The phase 3 trial is ongoing and is expected to complete enrollment in mid-2014, with data in mid-2015.
- **Conference call at 8:30am EST:** 877- 303-9216 (US) or (973) 935-8152 (OUS), passcode 51166480.
- **Reiterate Overweight rating.**

Overweight

PTCT, PTCT US

Price: \$15.32

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Biotechnology

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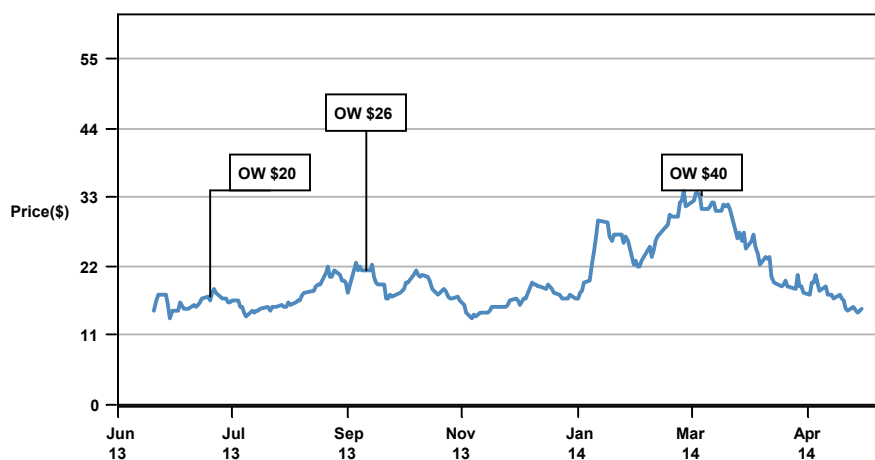
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PTC Therapeutics (PTCT, PTCT US) Price Chart



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

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