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# PTC Therapeutics (PTCT - OUTPERFORM): Analyst Day Highlights: Ataluren's Next Indication to be Determined in 2014, SMA Updates to Come in 2014 – Broad Pipeline Undervalued

**Price: $20.71** **12-Month Price Target: $55**

- **PTCT's Nov. 4th analyst day highlighted development of their pipeline beyond initial focus on ataluren in nmDMD and nmCF.** The update, by PTCT at their analyst day, highlighted work on additional indications for ataluren, their SMA candidate partnered with Roche, as well as a pipeline of promising oncology and antibacterial candidates.

- **SMA updates including a clinical path forward in 2014 are likely to drive near term value in PTCT.** We believe that as an orally bioavailable small molecule PTCT/Roche's SMA candidate is differentiated from the ISIS (ISIS:Not Covered) and PMO approaches for SMA. We highlight that ease and safety of systemic delivery of PTCT's compound may be preferential over intrathecally delivered compounds, and may better facilitate delivery very early in life, a time, during which animal models of SMA suggest maximum therapeutic benefit may be conferred.

- **Results presented for PTC/Roche's SMA oral-compound highlighted impressive systemic efficacy in SMA mouse models prolonging survival and reducing phenotypic abnormalities.** In addition to demonstrating a dose dependent increase in SMN protein, PTCT showed that early therapy (day-0) with their compound appears to result in optimal restoration of near-normal life span and phenotypic characteristics (Figure 1). Demonstration of systemic impact of their SMA candidate, imparted by excellent bioavailability, included prevention of tail necrosis as well as reduction in paw edema in SMA Type III mice. The company also highlighted, for the first time, their candidates ability to confer protection from neuromuscular junction denervation, with treated models showing 90-100% of normal junctions vs. untreated SMA models that demonstrated ~50% of near normal innervated junctions (Exhibit 2).

- **New indications for ataluren's clinical development are to be selected in 2014 and may offer rapid routes to registration supported by a 600-patient strong clinical data set (Figure 3).** The company highlighted work in MPS disorders where small elevation in enzymes, that are not normally produced in the disease state, could result significant and readily identifiable clinical benefit. We highlight that in rare lysosomal storage diseases surrogate markers of efficacy may support accelerated approval. Furthermore, it is likely that ataluren's safety, now demonstrated in 600 patients may support a rapid route to registration following as little as one additional trial.

- **The company presented new data for ataluren from their Phase IIb study highlighted additional clinical benefit in timed functioned tests for DMD.** PTCT presented a subset analysis in a decline phase subgroup that demonstrated that patients on low-dose ataluren demonstrated benefit in 10 meter run, climb and decline assays (Exhibit 4).

- **PTCT reviewed the multiple assay approach implemented to validate ataluren's ability to read-through non-sense mutations beyond initial luciferase assays (Figure 5).** The company also reviewed several internal and external clinical trials supporting ataluren's activity in several indications. Recall, several investigators have attempted, and failed, to reproduce PTCT's original (but not subsequent) assays supporting ataluren's ability to read-through non-sense mutations.

- **Anticipated near-term progress in PTCT's pipeline that could drive valuation include, EU market launch of ataluren in 2014, selection of ataluren's next indication, and the start of potentially registration-worthy Phase I/II studies for PTCT's SMA candidate that is partnered with Roche.** We view PTCT with an enterprise value of ~$300 as undervalued relative to companies at similar stages of development with drugs for rare diseases.

- **We reiterate our OUTPERFORM rating and 12-month price target of $55/share.** Our $55 price target is derived by applying an 8X multiple to estimated 2017 revenues for ataluren in nmDMD and nmCF, discounted 25% and 35% annually, respectively. Conditional approval of ataluren in the EU and success of the SMA candidate remain upside to our price target.

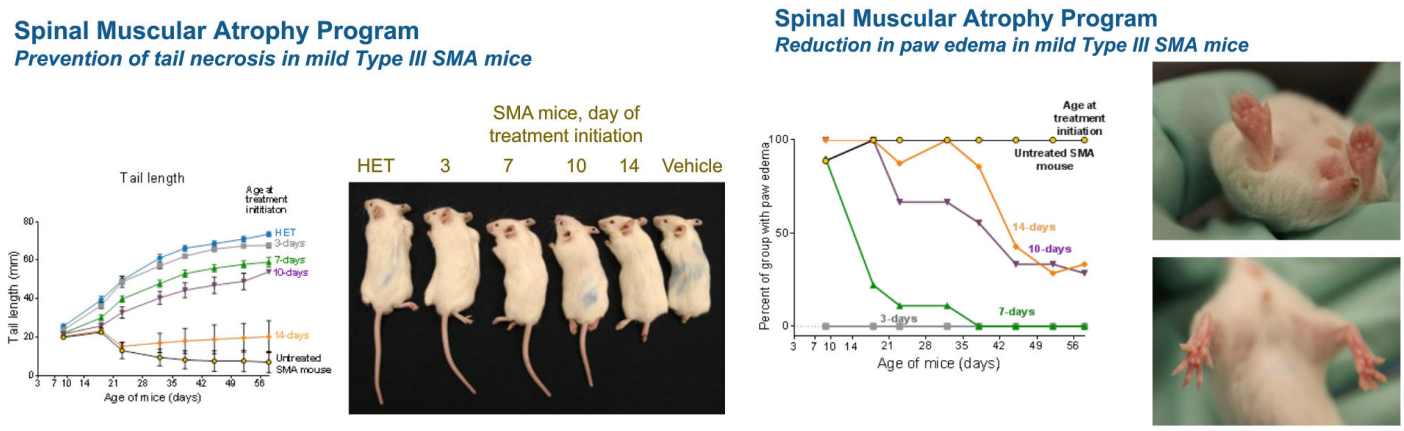
- Risks to the attainment of our price target include 1) failure of ataluren in the clinic in DMD or CF; 2) regulatory failure of ataluren; and 3) inability to fund the development or execute on the commercializing of ataluren globally.

- **PTCT's oncology candidate PTC596 targets BMI1 protein expression that is thought to have a role in cancer stem cells and has displayed both single agent activity and synergy with chemotherapeutics.** Early data suggests significant activity in liquid and solid tumors. IND-enabling safety studies are on going, with further development first in GBM as a possible primary indication.

- **The company has also developed a novel class of novel antibacterial agents based upon 2-pyridone chemical scaffold (Figure 6).** The company is looking to first develop candidate PTC-847 for the treatment gonorrhea, an area of significant unmet need, rapid and wide-spread resistance. We believe that further development of these compounds may be suitable for out-licensing or partnering.

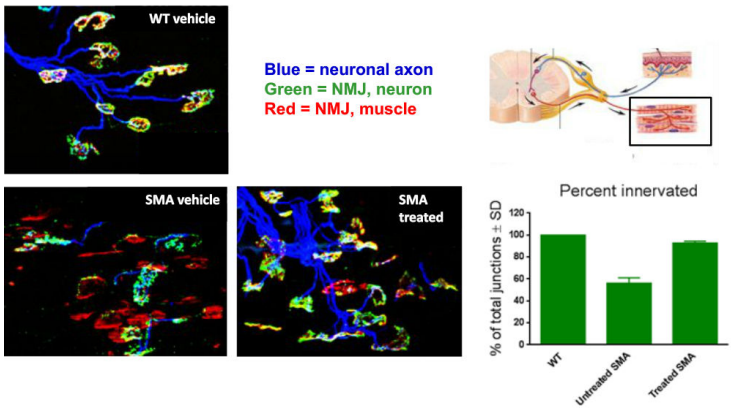
- **The company also highlighted their GEMS technology targeting post-transcriptional regulation gene expression (Figure 7).** PTCT's GEMS platform has the ability elucidate molecules that post-translational processed including non-sense suppression (ataluren), alternative splicing (SMA program, DMD program), nucleotide-repeat disease, non-sense mediated decay and post-translational production. Specifically, the company noted they can target their platform to design drugs for nucleotide-repeat diseases including, myotonic dystrophy, Huntington's disease, among other diseases cased by nucleotide repeat expansions with significant unmet medical need.

**Figure 1: Systemic effect of PTC/Roche's SMA candidate in mouse models**




*Source: Company data, Wedbush Securities, Inc.*

**Figure 2: SMA candidate protects from neuromuscular junction denervation**




*Source: Company data, Wedbush Securities, Inc.*

**Figure 3: Ataluren has demonstrated activity in multiple disease models**

**Muscle disease**
- DMD (Welch 2007, Kayali 2012, Arners 2013 [submitted])
- Miyoshi Myopathy (Wang 2010)

**Neurological disorders**
- Infantile Neuronal Ceroid Lipofuscinoses (INCL) (Sarkar 2011, Miller 2013)
- Late Infantile Ceroid Lipofuscinoses (LINCL) (Miller 2013)
- Ataxia telangiectasia (Du 2013)
- Usher Syndrome (USCH1C) (Goldmann 2011, Goldmann 2012)

**Ion channel disease**
- Cystic fibrosis (CF) (Du 2008, Kerem 2008, Sermet-Gaudelus 2010, Wilshanski 2011, Gonzalez-Hilarion 2012)

**Skin disease**
- Pseudoxanthoma Elasticum (Zhou 2013)

**Eye disorders**
- Choroideremia (Moosajee, unpublished)
- Aniridia (Gregory-Evans, unpublished)

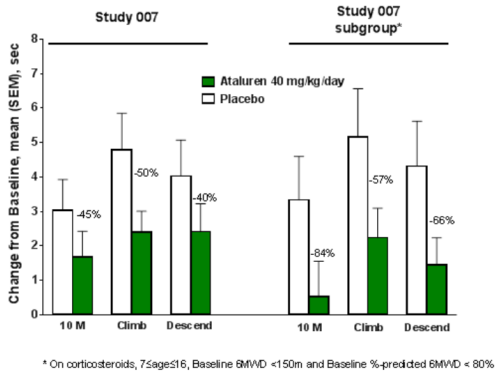
**Pulmonary disease**
- Heritable pulmonary arterial hypertension (HPAH) (Drake 2013)

**Metabolic disorders**
- Carnitine Palmitoyltransferase 1A Deficiency (Tan 2011)
- Methylmalonic Aciduria (MMA) (Buck 2010)
- Propionic Acidemia (PA) (Sanchez-Alcudia 2012)
- Maroteaux-Lamy syndrome (MPS VI) (Bartolomeo, 2013)
- Hurler's syndromes (MPS I) (Keeling, unpublished)

Studies from independent investigators

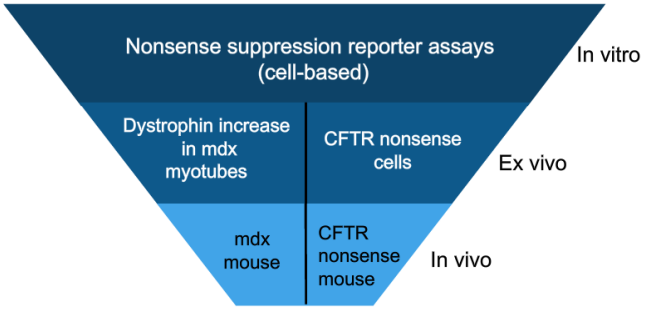
*Source: Company data, Wedbush Securities, Inc.*

**Figure 4: Ataluren demonstrated activity in timed function tests – secondary endpoints in their Phase IIb trial in nmDMD**



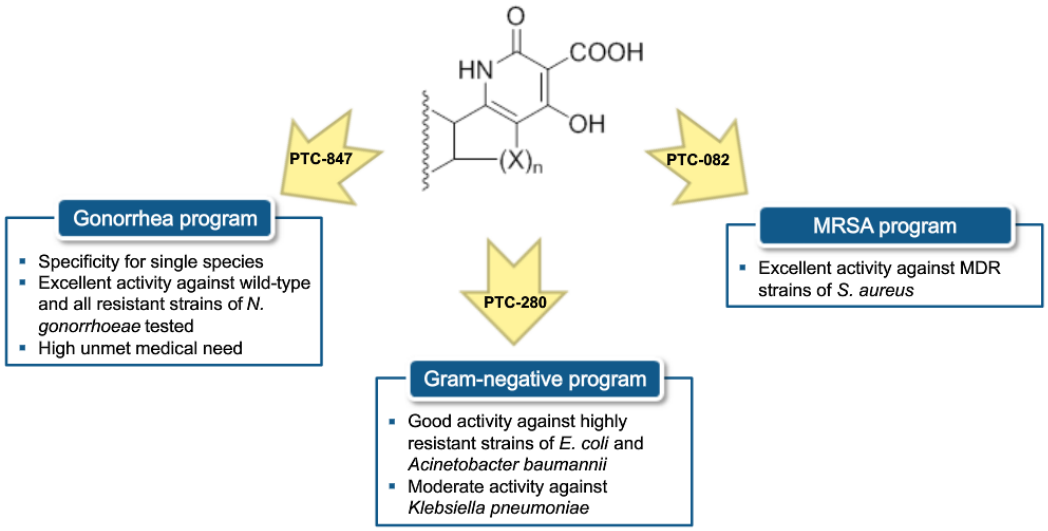

*Source: Company data, Wedbush Securities, Inc.*

**Figure 5: Screening Tier Used to Ataluren**



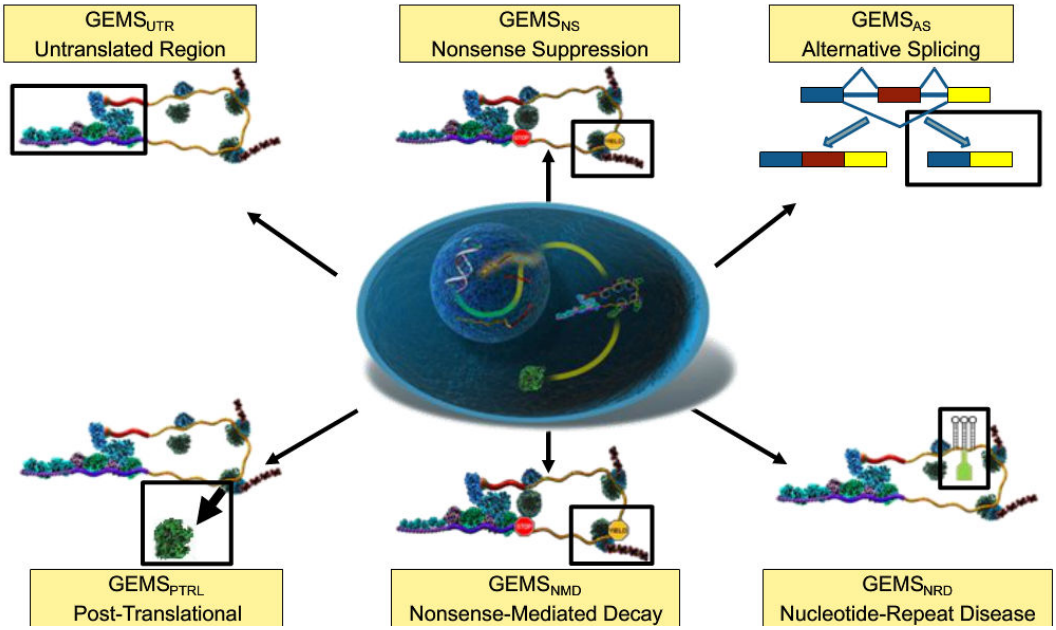

*Source: Company data, Wedbush Securities, Inc.*

**Figure 6: PTC's novel chemical scaffold may produce several antibacterial agents**




*Source: Company data, Wedbush Securities, Inc.*

**Figure 7: PTC GEMS Technology Platform -Targets Post-Transcriptional Regulation**




*Source: Company data, Wedbush Securities, Inc.*

## Product Pipeline

| *Candidate* | *Stage of Development* | *Next Event* |
|---|---|---|
| Ataluren nmDMD | Phase III | Full enrollment H1:14 |
| Ataluren nmCF | Phase III | Full enrollment in Phase III trial in nmCF |
| Ataluren (next rare disease) | N/A | Election of candidate in 2014 |
| SMA | IND | Initiation of Phase I/II clinical studies 2014 |
| PTC596 | IND | |
| Antibacterial Compound | Lead optimization | |

## Investment Thesis

PTC is a biotechnology company focused on the development of ataluren, a molecularly targeted, orally delivered treatment for rare and ultra-rare diseases, including Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations. Ataluren may also be effective in treating 2500 other rare diseases and certain cancers caused by nonsense mutations since its mechanism of action is broadly applicable to these molecular lesions. PTC is also developing a candidate for spinal muscle atrophy a fatal and rare disease that most severely impacts infants. We believe that ataluren will be shown to be safe and efficacious in on going Phase III trials in nmDMD as well as those set to begin (H1:14) in nmCF. In our opinion, the Street is overly discounting their lead Phase III program for ataluren in nnDMD, which showed mixed results, overall in a Phase II trial, but positive trends and nominal statistically significant benefit in the current subgroups being evaluated in the Phase III trial. We believe that, given lessons learned from prior pioneering trials of ataluren in DMD, PTC has enriched their Phase III trial for success and that it is highly likely to show positive results in mid:2015. Additionally, PTC may also receive conditional approval for ataluren for nmDMD in the EU by YE:13, a scenario which would result in an early 2014 launch and would $20 in upside to our $55, 12-month price target. Final read-outs from a soon to be initiated (H1:14) trial for ataluren CF are anticipated by mid:16. PTC's SMA program, partnered with Roche, remains additional upside to our estimates. We believe that this program likely be accelerated through the clinic due to significant unmet medical need in this devastating disease and that breakthrough results in Phase I/II trials could form the basis for a registration filing as early as 2015.

## Upcoming Milestones

| | |
|---|---|
| H2:13 | Seek early access programs (in select territories) for ataluren in nmDMD |
| Q4:13 | MAA filing for conditional approval of ataluren for nmCF in the EU |
| YE:13 | Potential conditional approval of ataluren for nmDMD in the EU |
| H1:14 | Full enrollment in the confirmatory Phase III trial of ataluren in nmDMD |
| Q1:14 | Initiation of a Phase III trial of ataluren in nmCF |
| H1:14 | Bring anti-infective asset forward for gonorrhea |
| H2:14 | Potential data from the Phase IIb open-label extension study in the EU |
| YE:14 | Potential conditional approval of ataluren for nmCF in the EU |
| 2014 | Initiation of Phase I/II trials of SMN2 candidate for SMA |
| H1:15 | Completion of the confirmatory Phase III trial of ataluren in nmDMD |
| H2:15 | FDA and MAA filing for full approval of ataluren for nmDMD |
| H2:15 | Completion of the confirmatory Phase III trial of ataluren in nmCF |
| 2015 | Potential accelerated approval of candidate for SMA |
| H1:16 | FDA and MAA filing for full approval of ataluren for nmCF |

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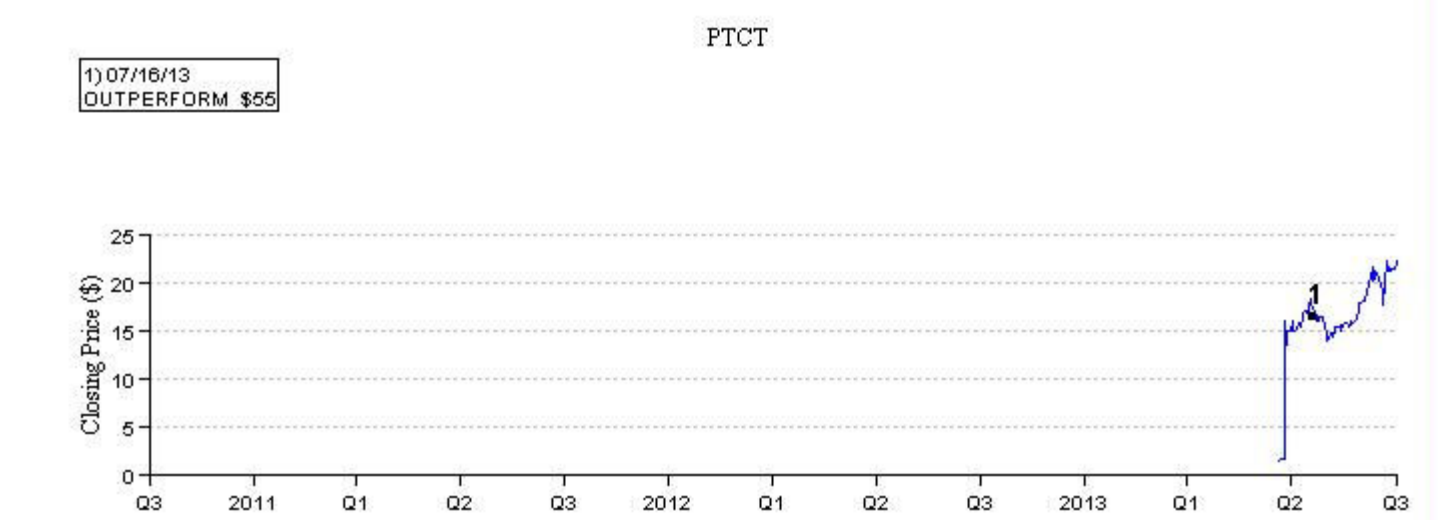


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