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PTC Therapeutics (PTCT - OUTPERFORM): An Appropriately Powered Study in DMD Is Only the Beginning - Looking for SMA Update and Ataluren's Next Indication in 2014 - Reiterate OUTPERFORM

Price: $16.42 **12-Month Price Target: $55**

- **Recent weakness in PTCT shares on the FDA's cautionary statements regarding Sarepta's (SRPT:OUTPERFORM) trials for ataluren have been misinterpreted, in our view.** Tuesday morning on SRPT's Q3:13 earnings call it was announced that the FDA is no longer willing to consider an NDA submission for eteplirsen based on current data. The agency also expressed concerns regarding the viability of running reasonably powered well controlled studies using the 6MWT in DMD.

- **It appears to us that a key FDA statement regarding Phase III trial designs for drugs for DMD has been taken out of context by the Street.** Specifically the FDA appears to stand by 6MWT as an endpoint, we quote "We stress that we would still accept 6MWT in an appropriately powered study…" We believe that PTCT's Phase III trial design for ataluren represents the model of "appropriate powered study" (Study design parameters Figure 1) and that the FDA's statement is a positive for the company. PTCT's 220 patient Phase III trial of ataluren in nmDMD is >85% powered to detect a 30m difference in 6MWD vs. placebo. Importantly we highlight that the FDA is most concerned about a statistically significant $p<0.05$ outcomes and that even a 20m difference in 6MWD vs. placebo would be acceptable.

- **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.

- **Recall that 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's data showed that early therapy (day-0) with their compound appears to result in optimal restoration of near-normal life span and phenotypic characteristics. Furthermore, 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. Studies also suggest that the candidate is able 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.

- **Anticipated near-term progress in PTCT's pipeline that could drive valuation include, 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 <$300M as undervalued relative to companies at similar stages of development with drugs for rare diseases.

- **PTCT reports its Q3:13 on Thursday, November 14, 2013 at 8am ET.**

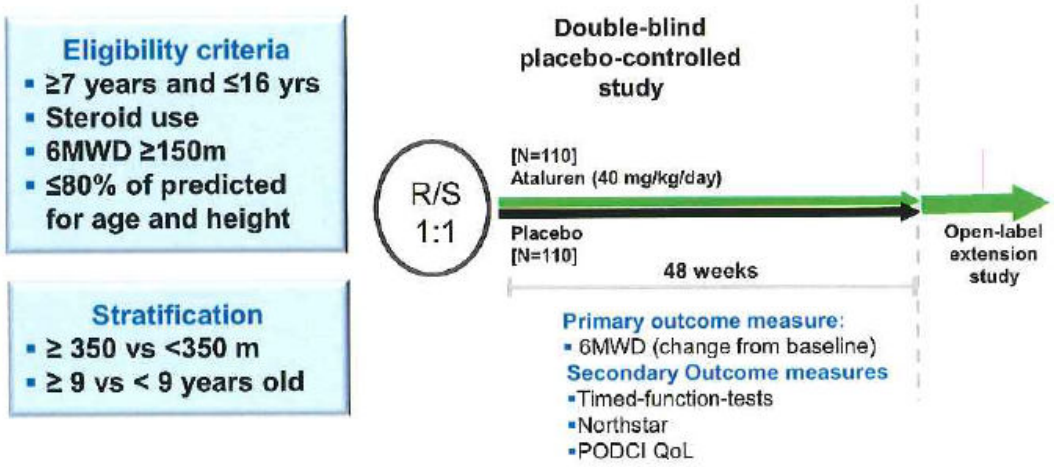
- **We reiterate our OUTPERFORM rating and 12-month price target of $55/share.** Our $55 price target is derived by applying an 8X multiple to estimated 2017 revenues for ataluren in nmDMD and nmCF, discounted 25% and 35% annually, respectively. Conditional approval of ataluren in the EU and success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

Upcoming Milestones

H2:13	Preparations for early access programs (in select territories) for ataluren in nmDMD
Q4:13	MAA filing for conditional approval of ataluren for nmCF in the EU
Dec. 17, 2013	Lockup expiration
YE:13	Potential conditional approval of ataluren for nmDMD in the EU
Mid: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
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

Figure 1: PTCT's Phase III Trial Design for Ataluren in nmDMD



Criteria	Phase III
Patients	~220 (1:1 Placebo Controlled)
Dose	10,10,20 mg/kg (Low Dose)
Primary Endpoint	Change in 6MWD from Baseline
Age	≥7 and ≤16 years old (onset by age of 6)
Baseline 6MWD	≥150 m
Gower's Maneuver	Possible inclusion criteria (<7 seconds)
%-Predicted 6MWD	≥80%
Steroid Use	Us is required minimum 6-months prior to trial start
Powering	>85% Powered to Detect 30m Difference in 6MWD

Source: Company data, Wedbush Securities, Inc.

Covered Companies Mentioned Table

Name	Ticker	Rating	Price Target	Current Price
Sarepta	SRPT	OUTPERFORM	$45	$13.16

Analyst Certification
I, Christopher N. Marai, Ph.D., Gregory R. Wade, Ph.D., David M. Nierengarten, Ph.D., certify that the views expressed in this report accurately reflect my personal opinion and that I have not and will not, directly or indirectly, receive compensation or other payments in connection with my specific recommendations or views contained in this report.

Disclosure information regarding historical ratings and price targets is available at http://www.wedbush.com/ResearchDisclosure/DisclosureQ313.pdf

Investment Rating System:
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Neutral: Expect the total return of the stock to perform in-line with the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.
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The Investment Ratings are based on the expected performance of a stock (based on anticipated total return to price target) relative to the other stocks in the analyst's coverage universe (or the analyst's team coverage).*

Rating Distribution (as of September 30, 2013)	**Investment Banking Relationships (as of September 30, 2013)**
Outperform:55% Neutral: 41% Underperform: 4%	Outperform:14% Neutral: 2% Underperform: 0%

The Distribution of Ratings is required by FINRA rules; however, WS' stock ratings of Outperform, Neutral, and Underperform most closely conform to Buy, Hold, and Sell, respectively. Please note, however, the definitions are not the same as WS' stock ratings are on a relative basis.

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Wedbush Equity Research Disclosures as of November 12, 2013

Company	Disclosure
PTC Therapeutics	1,3,5
Sarepta Therapeutics	1,3,4,5

Research Disclosure Legend
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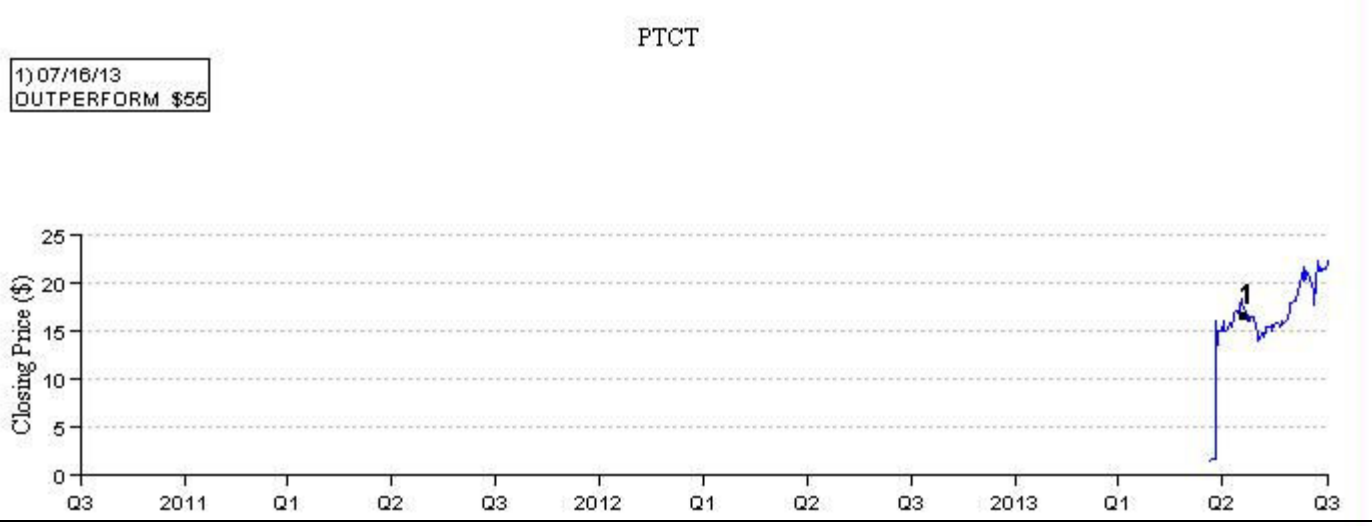

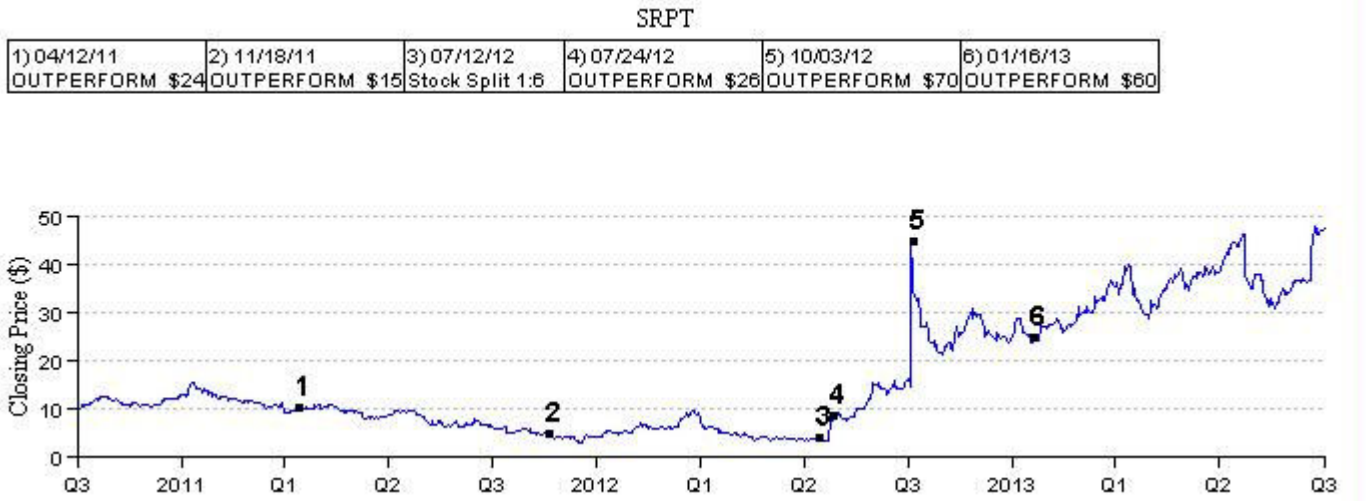

* WS changed its rating system from (Strong Buy/Buy/Hold/Sell) to (Outperform/ Neutral/Underperform) on July 14, 2009.
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