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PTC Therapeutics (PTCT - OUTPERFORM): Sell-off Unfounded - PTCT Is an Undervalued and Misunderstood Low-Risk Name with Catalysts Ahead - Reiterate OUTPERFORM

Price: $17.43 **12-Month Price Target: $55**

- **Recent weakness in PTCT shares is unfounded and represents a buying opportunity, in our view.** Discussions with KOL's at WMS highlighted enthusiasm for ataluren for DMD and PTCT/Roche's product for SMA. We believe the Street has erroneously compared PTCT to RNA and that fallout from the failed drisapersen Phase III trial has at least partially tempered interest in the space. We note that PTC's drug works by a completely different mechanism, has better bioavailability, safety data in more than 600 patients and has demonstrated clinically meaningful benefit in large (174 patient) Phase IIb trials.

- **The upcoming analyst day on Oct. 25 could result in >20% appreciation in PTCT, in our opinion, as investors become more fully appraised of ataluren's risk reduced Phase III's, the opportunity in SMA and the rest of PTCT's pipeline including oncology candidates.** PTCT's SMA candidate, partnered with Roche, could be discussed at the analyst day, highlighting value beyond ataluren and a potentially rapid clinical program. We believe that a signal of efficacy in even a small number of SMA patients could prove worthy of approval in this fatal disease and that PTCT's oral candidate could offer patients options and potentially better efficacy when compared to ISIS's (ISIS:not covered) intrathecally administered candidate for SMA.

- **PTCT's Phase III studies of ataluren in DMD and CF are enriched for success and supported by substantial safety data in more than 600 patients.** PTC's Phase III designs for ataluren take advantage of substantial data-sets from prior studies of ataluren, allowing the company to select patients most likely to benefit from drug, which we believe enhances their chances for success. Ataluren has been shown to be safe and tolerable in more than 600 patients, is specific for pre-mature stop-codons (caused by non-sense mutation) and does not result in read-through of normal stop-codons or overt toxicities.

- **With an enterprise value of ~$260M, PTCT is undervalued relative to orphan-disease comparables, in our opinion (Table 1).** PTCT has three large trials of supportive data that we believe is highly supportive of ataluren's benefit in DMD and CF and is largely being overlooked by the Street. We also believe that no value has been assigned to a potential EU launch of ataluren for DMD in the EU in 2014. PTCT currently has ~$165M in cash.

Table 1: Rare Disease Comparable Companies to PTCT

Company	Ticker	MC (M)	Stage	
NPS Pharma	NPSP	$3,100	Market Launches US/EU +	NPSP: Outperform
Aegerion	AEGR	$2,400	Market Launches US/EU +	AEGR: Not Covered
Synageva	GEVA	$1,800	Phase III Enrolling	GEVA: Neutral
Sarepta Therapeutics	SRPT	$1,700	Phase IIb	SRPT: Outperform
Raptor Pharma	RPTP	$810	Market Launches US/EU	RPTP: Neutral
Prothena	PRTA	$440	Phase I	PRTA: Outperform
PTC Therapeutics	**PTCT**	**$420**	**Phase III Enrolling/Potential Launch 2014**	**PTCT: Outperform**

Source: Wedbush Securities

- **The marketing of ataluren in the EU in 2014 remains a free-call option that is not priced into the stock, in our opinion.** Given no other therapeutic options and a relatively benign safety profile, we believe that the benefit risk profile favors ataluren's approval (initiation report). Discussions with key leaders of PTCT's commercialization strategy at WMS reveal that launch plans for the EU are well underway. PTCT expects a decision from EU regulators on a potential conditional approval by YE:13 and also has the option to sell the drug on a "named patient basis" at typical orphan drug prices in the EU should conditional approval not occur. Our checks reveal that EU regulators are mostly concerned about the ability to enroll a Phase III trial should conditional approval occur.

- **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 .

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 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	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

Ataluren's Broad Mechanism May Be Applicable to 2400 Diseases Caused by Non-Sense Mutations

Ataluren is an oral, small-molecule, with systemic bioavailability that induces dystrophin production by a translational read-through mechanism. We note that ataluren's molecular properties and anticipated mechanism of action are substantially different from drisapersen or eteplirsen, both of which are large oligonucleotides (20+-mer) that function by an exon-skipping mechanism. Additionally, ataluren treats of a sub-type of Duchenne muscular dystrophy caused by non-sense mutations that result in pre-mature stop codons and incomplete dystrophin translation and thus does not compete with Sarepta's (SRPT: OUTPERFORM) eteplirsen or Prosensa (RNA:NEUTRAL) drisapersen. Ataluren is currently in clinical trials for ataluren and cystic fibrosis, the company is investigating additional opportunities in other diseases caused by non-sense mutations in which the read-through mechanism is anticipated to work.

Ataluren's Data Set Is Large and Highlights Impressive Safety and Efficacy

In Phase IIb trials, ataluren demonstrated a 29.7 m benefit in 6MWD vs. placebo (nominal p=0.0584) in patents with DMD caused by a non-sense mutation. We note that this low-dose cohort was predefined as part of the primary endpoint of the Phase IIb study design and that ataluren's demonstrated benefit is independent of any post-hoc data analysis. Additionally, when looking at a pre-defined supportive endpoint, time to persistent 10% worsening, 26% of patients in the low-dose ITT population progressed compared to 44% on placebo (nominal p=0.039).

Covered Companies Mentioned Table

Company	Ticker	Rating	Price Target	Price (close 10/10/13)
NPS Pharma	NPSP	OUTPERFORM	$43	$33.15
Synageva	GEVA	NEUTRAL	$67	$58.91
Sarepta Therapeutics	SRPT	OUTPERFORM	$60	$47.18
Raptor Pharma	RPTP	NEUTRAL	$10	$13.58
Prothena	PRTA	OUTPERFORM	$18	$21.00
PTC Therapeutics	PTCT	OUTPERFORM	$55	$17.43

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.

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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.
Underperform: Expect the total return of the stock to underperform relative to the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.

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 June 30, 2013)	**Investment Banking Relationships (as of June 30, 2013)**
Outperform:54% Neutral: 41% Underperform: 5%	Outperform:15% Neutral: 1% Underperform: 0%

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Wedbush Equity Research Disclosures as of October 11, 2013

Company	Disclosure
PTC Therapeutics	1,3,5,7
NPS Pharmaceuticals Inc.	1,3,7
Sarepta Therapeutics	1,3,4,5,7
Synageva BioPharma Corp.	1,3,4,5,7
Raptor Pharmaceutical	1
Prothena Corp.	1,3,5,7

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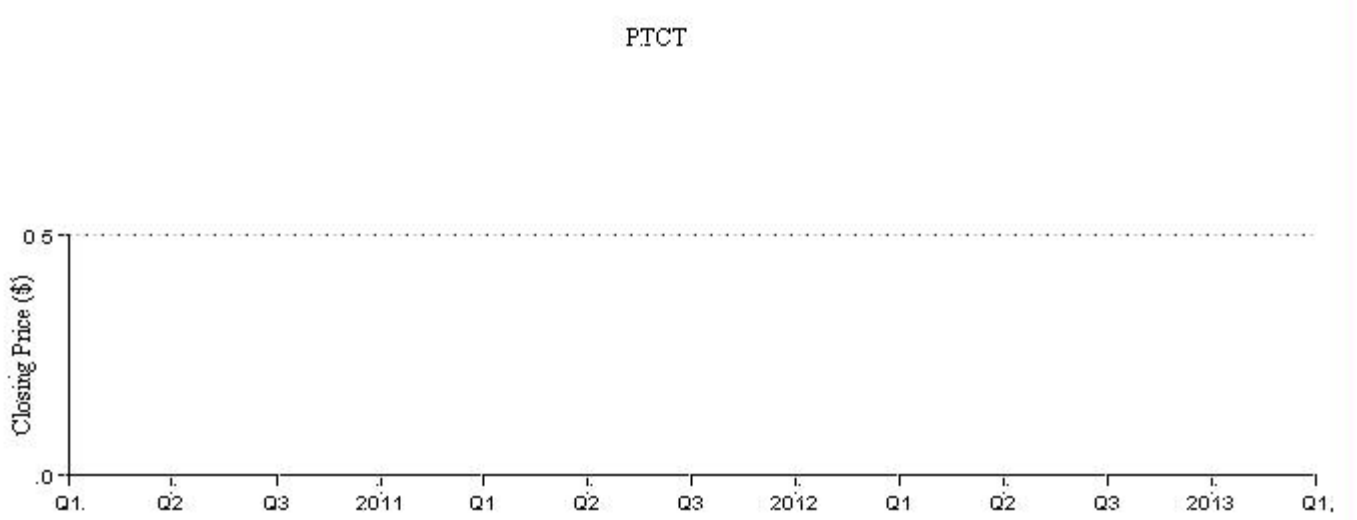

* WS changed its rating system from (Strong Buy/Buy/Hold/Sell) to (Outperform/ Neutral/Underperform) on July 14, 2009.
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