WEDBUSH QUICK NOTE

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September 23, 2013

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PTC Therapeutics (PTCT - OUTPERFORM): Drisapersen Results Highlight Ataluren's Clinically Meaningful Benefit - EU Conditional Approval is Now More Likely, in Our Opinion - Upside in SMA

Price: $17.88 **12-Month Price Target: $55**

- **Weakness in PTCT following surprisingly negative results from Prosensa (RNA: NEUTRAL) represents a buying opportunity, in our view.** We believe that unanticipated and disappointing drisapersen Phase III results reported on Friday, highlight the difficulties associated with developing drugs for DMD and further justify another look at the opportunity for PTCT's candidate, ataluren.

- **Ataluren is an orally available, 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 RNA's drisapersen.

- **In Phase IIb trials, low-dose 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).

- **We believe that at PTCT's current valuation, the Street is largely overlooking evidence of ataluren's efficacy and the near-term market opportunity for the drug in the EU.** We believe that this result, a near 30-meter benefit, will be looked upon favorably by EU regulators, particularly given what is now known about the heterogeneity of the disease, the cohort enrolled in the trial and ataluren's dose response. Recall that investigators broadly believe that a 30-meter benefit is considered a clinically relevant benefit in DMD.

- **We anticipate that ataluren will be marketed in the EU in 2014 and believe that drisapersen's surprising Phase III results increase the likeliness that some leniency is prudent in consideration for ataluren's conditional approval, given the difficulty in developing treatments for the disease.** Given no other therapeutic options and a relatively benign safety profile, we believe that the benefit risk profile favors ataluren's approval (data discussed above and in initiation report). In particularly, we highlight that the vast majority of experts in DMD did not anticipate results in the Phase III GSK/Prosensa study, making it likely that the community will need to step back to reassess expectations of new therapies with respect to benefit and acceptable risks for patients with DMD. Further, we believe that planned YE:13 submission for conditional approval for ataluren in nmCF in the EU suggests conversations regarding ataluren in DMD with the EMA have been positive.

- **Wedbush will host an investigator dinner with Dr. Craig McDonald, expert on 6MWT endpoint and DMD trial clinician, on October 2 at World Muscle Society (WMS) – contact your sales person for further details.** We anticipate that we may gain insights into a clinician's experience with ataluren, drisapersen and eteplirsen. We hope to gain a clinician's perspective on top-line results presented for Prosensa, and will discuss interpretation of the results expected for eteplirsen, drisapersen and PRO044.

- **Anticipated near-term progress in PTCT's pipeline that could drive valuation include, conditional approval for ataluren in the EU, an IND filing and the start of potentially registration-worthy Phase I/II studies for PTCT's SMA candidate that is partnered with Roche.**

- **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 Events of Interest Related to Drug Development in DMD

Sept. 25-27	DIA/FDA Oligonucleotide Conference – SRPT Data, RNA/GSK Data Update	(Washington, DC)
Oct 2.	Wedbush Expert Dinner – Dr. Craig McDonald and other experts in DMD at World Muscle Society	(Asilomar, CA)
Oct 3.	SRPT Management Event at World Muscle Society (WMS) – Expert panel in DMD	(Asilomar, CA)
Oct 5.	Phase III data presentation for drisapersen	(Asilomar, CA)

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
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

Covered Companies Mentioned Table

Company Name	Ticker	Rating	Price Target	Last Trade Price
Sarepta	SRPT	Outperform	$60	$43.30
Prosensa	RNA	Neutral	$7	$7.14
PTC Therapeutics	PTCT	Outperform	$55	$17.88

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/DisclosureQ213.pdf

Investment Rating System:
Outperform: Expect the total return of the stock to outperform relative to the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.
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%

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 September 23, 2013

Company	Disclosure
PTC Therapeutics	1,3,5,7
Sarepta Therapeutics	1,3,4,5,7
Prosensa Holding N.V.	1,3,5,7

Research Disclosure Legend
1. WS makes a market in the securities of the subject company.
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3. WS co-managed a public offering of securities within the last 12 months.
4. WS has received compensation for investment banking services within the last 12 months.
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6. WS is acting as financial advisor.
7. WS expects to receive compensation for investment banking services within the next 3 months.
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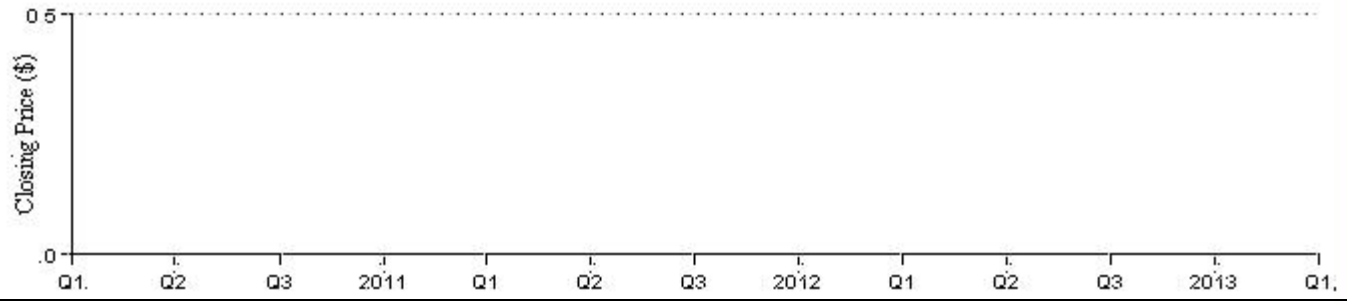
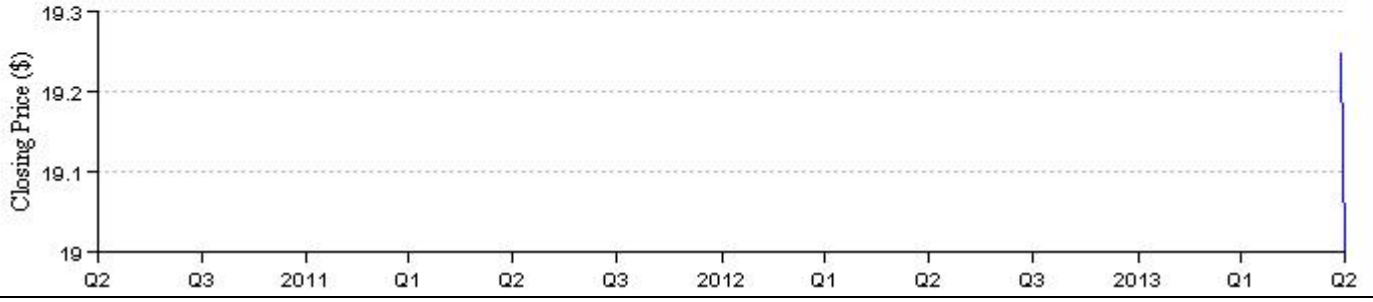
PTCT



RNA



SRPT

1) 04/12/11 OUTPERFORM $24	2) 11/18/11 OUTPERFORM $15	3) 07/12/12 Stock Split 1:6	4) 07/24/12 OUTPERFORM $26	5) 10/03/12 OUTPERFORM $70	6) 01/16/13 OUTPERFORM $60

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