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August 8, 2013

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PTC Therapeutics (PTCT - OUTPERFORM): SMA Collaboration $10M Milestone Achieved - PTCT Remains Undervalued and Misunderstood - Reiterate OUTPERFORM

Price: $15.08 **12-Month Price Target: $55**

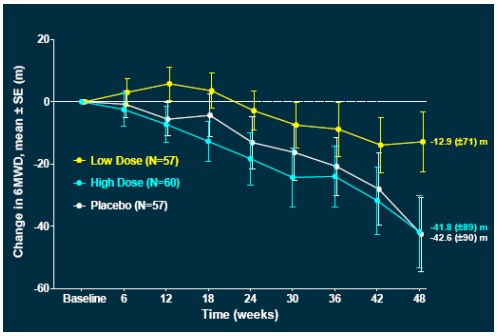
- **PTCT announced the selection of their lead candidate for SMA and received a $10M milestone payment from partner Roche.** PTC's oral, small molecule candidate has been shown to modify splicing at very low concentrations in non-clinical studies involving cells from patients with spinal muscular atrophy. It was also been shown to increase both the inclusion of exon 7 in the SMN2 mRNA and the levels of SMN protein produced by SMN2. PTCT's SMA program remains upside to our price target and we note that positive clinical results in even just a few children could be sufficient for a regulatory filing in the SMA setting.

- **PTC's oral candidate for the treatment of SMA has been shown to extend the lifespan of SMA mice by 800%.** In mouse models of SMA that only the SMN2 gene, PTC's candidate has been shown to penetrate the blood-brain barrier and increase full-length SMN mRNA and protein in a variety of tissues. Additionally, treatment with this compound resulted in a survival benefit, restoration of body weight, prevention of motor neuron loss and improved motor function in mouse models. Additionally, we believe that the oral route of administration compares favorably to other SMA therapies in development that require intrathecal administration.

- **The next catalyst for PTCT remains an update on their conditional approval filing for ataluren in the EU.** Conditional approval of ataluren remains upside to our price target. We note that it is likely that ataluren will be available to patients in the EU in 2014 either following conditional approval or on a named patient basis. PTCT will report their Q2, Aug. 12 at 16:30 ET.

- **We believe that with an enterprise value of <$180M 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 the Street has largely overlooked Phase IIb data that showed low-dose ataluren resulted in a 29.7-meter benefit vs. placebo in 6MWD at 48-weeks. 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*. 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.

Figure 1: Ataluren Demonstrated 29.7m Benefit in 6MWD in the Pre-Defined Low Dose Cohort at 48-weeks



Source: Company data, Wedbush Securities, Inc.

- **PTCT will be presenting at the Wedbush Life Sciences Management Access Conference, Aug. 13 and will join us in a lunch panel discussion "Leaders in DMD and Rare Diseases" along with RNA, SRPT & Summit (SUMM:AIM).**

- **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 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.

Valuation Methodology

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 by YE:13 remains upside to our price target and would yield a 12-month price target of $75/share. We project that approval and commercialization of ataluren could generate ~$550 million in annual worldwide revenues in 2017 (our valuation year) in nmDMD and nmCF and potential peak global sales of >$1.5 billion. Success of the PTC's pre-clinical SMA candidate remains upside to our estimates. Similarly we arrive at our $55 price target by applying a 15x multiple to PTC's fully taxed EPS in 2017 discounted back 20% annually.

Upcoming Milestones

H2:13	Seek early access programs (in select territories) for ataluren in nmDMD
YE:13	Select clinical candidate for SMA program
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

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.

The analysts responsible for preparing research reports do not receive compensation based on specific investment banking activity. The analysts receive compensation that is based upon various factors including WS' total revenues, a portion of which are generated by WS' investment banking activities.

Wedbush Equity Research Disclosures as of August 8, 2013

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

Research Disclosure Legend
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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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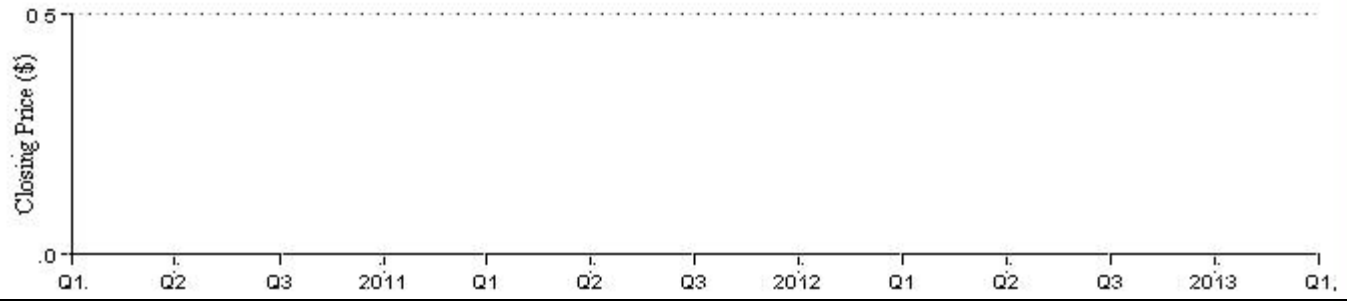
Wedbush disclosure price charts are updated within the first fifteen days of each new calendar quarter per FINRA regulations. Price charts for companies initiated upon in the current quarter, and rating and target price changes occurring in the current quarter, will not be displayed until the following quarter. Additional information on recommended securities is available on request.

PTCT



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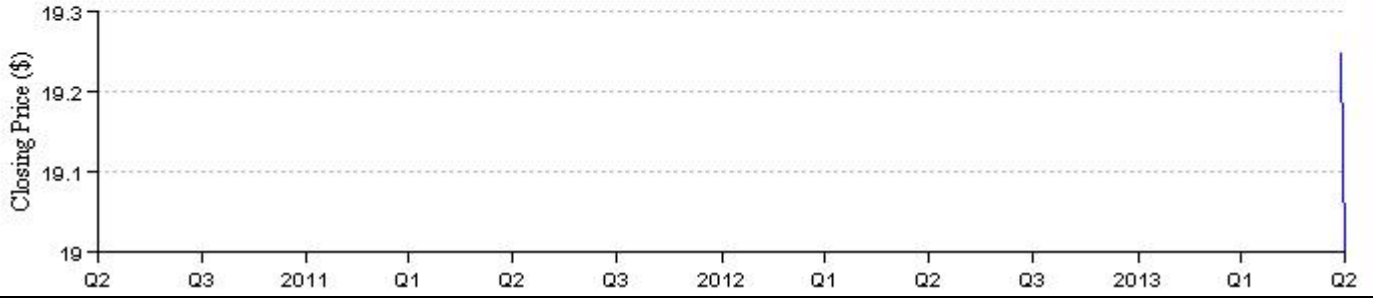

SRPT

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.
Please access the attached hyperlink for WS' Coverage Universe: http://www.wedbush.com/services/cmg/equities-division/research/equity-research Applicable disclosure information is also available upon request by contacting Ellen Kang in the Research Department at (213) 688-4529, by email to ellen.kang@wedbush.com, or the Business Conduct Department at (213) 688-8090. You may also submit a written request to the following: Business Conduct Department, 1000 Wilshire Blvd., Los Angeles, CA 90017.

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