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Recent FDA DMD Guidance May Put Pressure on EMA for Ataluren's Conditional Approval – ISIS's SMA Updates at AAN Informative to PTCT's Program

Price: $18.58 **12-Month Price Target: $55**

Ataluren for nmDMD

- **Sarepta (SRPT – OUTPERFORM) reported Monday an update from its interactions with the FDA regarding the potential accelerated approval of eteplirsen. We view an apparent greater receptiveness by the FDA to an NDA filing for eteplirsen as a potential positive for PTCT in itsinteraction with EU regulators over the conditional approval of ataluren for the treatment of nmDMD.** Recall, in January, the EMA adopted a negative opinion on conditional approval for ataluren, however, our checks suggest the CHMP is primarily concerned with PTCT's ability to enroll an ongoing Phase III trial should conditional approval be granted.

- **The next event for PTCT is an update on its appeal regarding a negative conditional approval opinion for ataluren in DMD in the EU potentially in Q2:14.** We see a positive opinion on an appeal as a free call, not priced in to the stock at current levels; it also represents upside to our current valuation.

- **We note that, regardless of a conditional approval decision by the EMA, it is possible that PTCT will market ataluren on a named patient/compassionate use basis in the EU and other territories in 2015**..

SMA Program

- **Upcoming data from a Phase II trial of ISIS-SMN$_{RX}$ at AAN (Abs S6.003) may inform the development of PTC/Roche's SMA candidate RG7800.** Recall, preliminary results for ISIS-SMN$_{RX}$ demonstrated dose-dependent benefit as measured by the HFMSE motor scale 9 months after the first dose. In particular, we anticipate further updates on the correlation between the potential surrogate endpoint of SMN protein levels and clinical benefit. ISIS Pharma (Not Covered) will hold an investor event to discuss ISIS-SMN$_{RX}$ Tuesday, April 29, 2014 at 6pm ET.

- **We note that PTCT/Roche's SMA Candidate RG7800 demonstrated a nearly 3-fold increase in SMN protein levels in the brain in early animal models, enough to nearly achieve healthy heterozygote protein levels**. Although slightly lower than the levels caused by ISIS-SMN$_{RX}$ in preclinical models, this is encouraging data for an oral candidate compared to ISIS' intrathecal injection. We highlight that the ease and safety of systemic delivery of RG7800 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.

- **Data from a Phase I trial of RG7800 in SMA by YE:14 may, in addition to safety and PK data, confirm mechanistic action through the measurement of SMN transcript levels in the blood plasma of healthy patients.** Recall that RG7800 is designed to increase the levels of full length SMN2 transcripts, and thus SMN protein levels, compared to the alternatively spliced SMN2 Δ7 transcript that produces dysfunctional protein. We would anticipate that decreased levels of SMN Δ7 transcript in blood plasma cells may be indicative of the drugs mechanistic activity in healthy volunteers.

Ataluren New Indications

- **PTCT intends to initiate trials for ataluren in new indications in 2014 with readouts ahead of the trial in DMD in 2015. We believe that with a significant safety database and strong pre-clinical evidence of efficacy these are derisked opportunities that will warrant additional valuation.** Based upon comps with molecularly targeted therapies such as EPZM (OUTPERFORM) we believe that two new indications (Aniridia and Hurler's (MPS I)) for ataluren could represent as much as $600M in MC in upside to PTCT. Published research provides promising evidence for ataluren's efficacy in Aniridia, a rare condition that severely limits the vision of ~5,000 pts in the U.S.

- **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. New indications for ataluren and the SMA candidate remain upside.

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 ongoing 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 nmDMD, 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. Final read-outs from a soon to be initiated (H1:14) trial for ataluren CF are anticipated by mid-2016. PTCT'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 Q1:14 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 PTC/Roche's SMA candidate RG7800 remains upside to our estimates. Similarly we arrive at our $55 price target by applying a 15x multiple to PTCT's fully taxed EPS in 2017 discounted back 20% annually.

Upcoming Milestones

April 29	ISIS: Data from a Phase II trial of ISIS-SMN_{RX} at the American Academy of Neurology (AAN) Meeting (Philadelphia, PA) (Abs S6.003)
Q2:14	Feedback from CHMP SAG meeting regarding request for reconsideration of conditional approval for ataluren for nmDMD in the EU
Q2:14	Potential MAA filing for conditional approval of ataluren for nmCF in the EU
Q2:14	Potential new opinion following a re-examination of the negative opinion regarding conditional approval of ataluren for nmDMD in the EU
Q2:14	Full enrollment in the confirmatory Phase III trial of ataluren in nmDMD
June 12-14	Update on SMA program (likely preclinical results) potentially at the International SMA Research Group Meeting, National Harbor, MD
H1:14	Initiation of a Phase III trial of ataluren in nmCF
H2:14	Potential data from the Phase IIb open-label extension study of ataluren in the EU
2014	Nomination of one or two new indications for ataluren (likely Aniridia and/or MPS I)
2014	Open label trial updates for ataluren in nmDMD at a scientific conference (US study safety only, EU efficacy at 0, 6,12,18 months)
YE:14	Potential conditional approval of ataluren for nmCF in the EU
YE:14/Q1:15	Potential start of proof-of-concept Phase II trials (potentially pivotal) for ataluren in one or two indications (ahead of ataluren in nmDMD)
YE:14/Q1:15	Potential top-line data and biomarker data from Roche/PTCT's Phase I healthy volunteers study of SMA candidate RG7800
H1:15	Top-line data from the confirmatory Phase III trial of ataluren in nmDMD
H2:15	FDA and MAA filing for full approval of ataluren for nmDMD
H2:15	Top-line data from the confirmatory Phase III trial of ataluren in nmCF
2015	Potential first commercial (named patient/compassionate use) sales of ataluren nmDMD in EU (or ROW) markets
2015	Potential initiation of pivotal Phase II/III trials RG7800 in patients with SMA
H1:16	FDA and MAA filing for full approval of ataluren for nmCF
2016	First commercial sales of ataluren following potential approval on Phase III data

Covered Companies Mentioned Table

Name	Ticker	Rating	Price Target	Current Price
Sarepta	SRPT	OUTPERFORM	$60	$33.98
Epizyme	EPZM	OUTPERFORM	$52	$20.01

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/DisclosureQ114.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 March 31, 2014)	**Investment Banking Relationships (as of March 31, 2014)**
Outperform:54% Neutral: 43% Underperform: 3%	Outperform:22% 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.

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 April 21, 2014

Company	Disclosure
PTC Therapeutics Sarepta Therapeutics Epizyme	1,3,4,5,7 1,3,4,5 1,3,4,5

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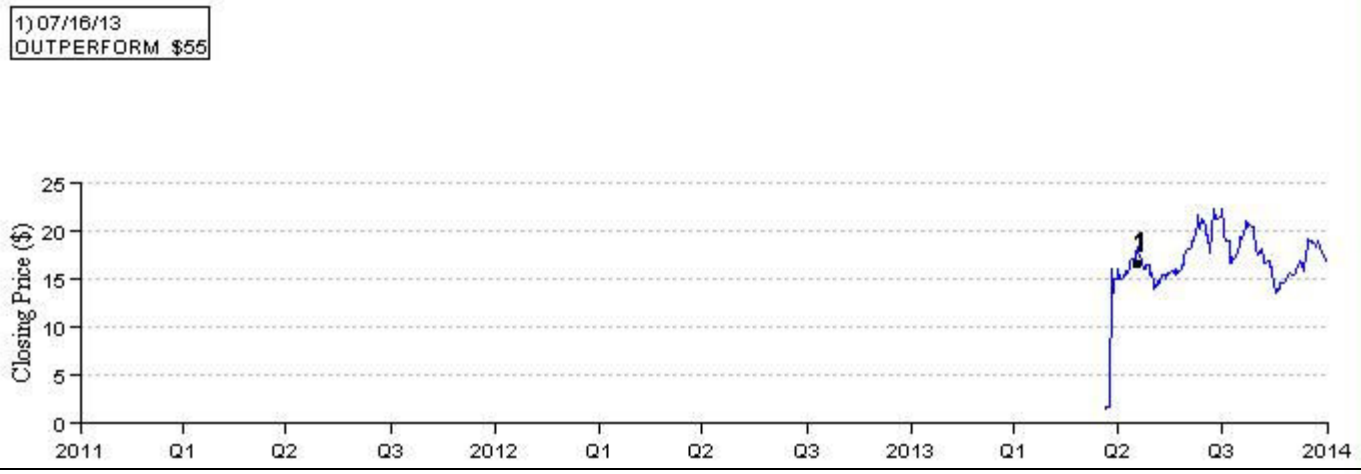
Price Charts
Wedbush disclosure price charts are updated within the first fifteen days of each new calendar quarter per FINRA regulations. Price charts for companies initiated upon in the current quarter, and rating and target price changes occurring in the current quarter, will not be displayed until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

PTCT



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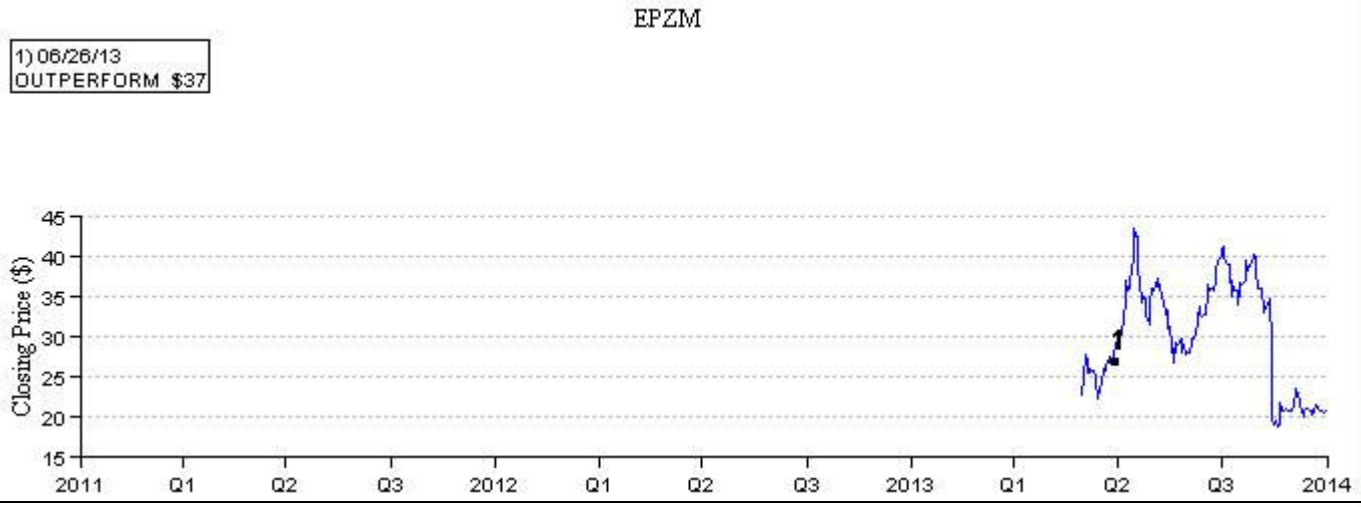
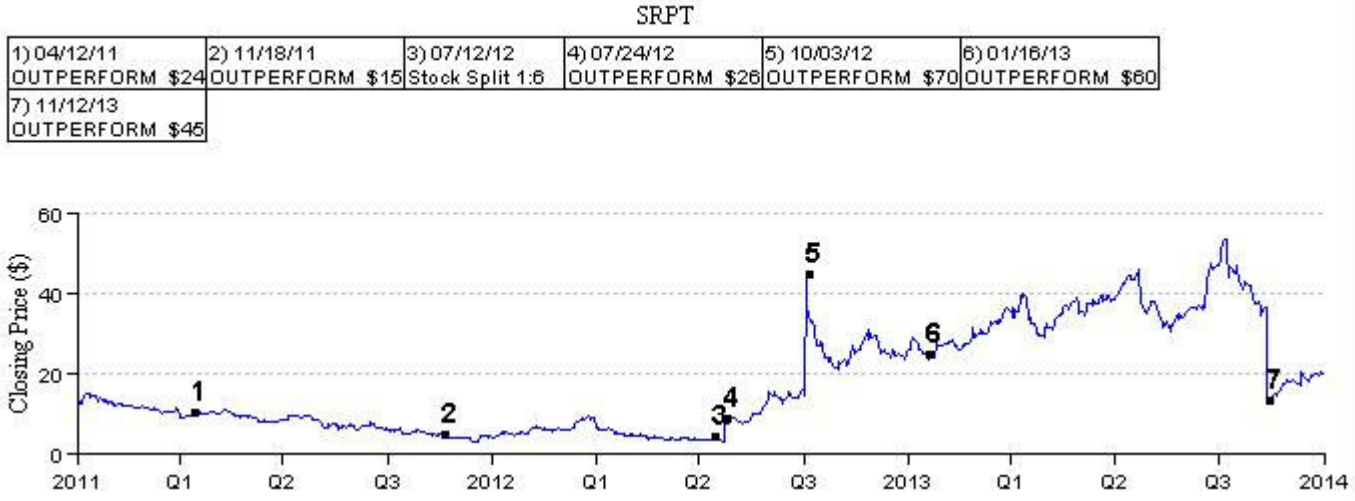


SRPT



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