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PTC Therapeutics (PTCT - OUTPERFORM): Negative Conditional Approval Opinion Lifts Overhang - Buy on Weakness: PIII Trial Enriched for Success - Reiterate OUTPERFORM and $55 PT

Price: $24.82 **12-Month Price Target: $55**

- **This morning, as anticipated, PTCT announced that the EMA adopted a negative opinion on its MMA for conditional approval of ataluren for nmDMD.** PTCT indicated that it intends to appeal the opinion. Under EMA rules, PTCT may request a re-examination of the recommendation within 15 days, and within 60 days, provide the reason for the request, the CHMP then has 60 days from receipt of the basis to consider the request re-examination, a final outcome is anticipated Q2:14. We believe that full enrollment in PTCT's PIII trial may be complete within the re-examination timeline and may form the basis for a change in opinion; however, our base case remains that full PIII results will be required prior to EMA approval of ataluren.

- **We believe that announcement of this opinion lifts an overhang on PTCT and we recommend aggressively accumulating shares of PTCT on weakness and higher volume in anticipation of several other catalysts for PTCT in 2014.** We believe shares of PTCT were broadly weak ahead of this outcome, which was anticipated to be negative, and that this announcement relieves an overhang on the shares. We anticipate that the next catalysts for PTCT shares will include an update on full enrollment in the Phase III trial of ataluren in nmDMD and additional SMA data updates. PTCT is also expected to announce a new indication for ataluren in 2014 as well as the start of enrollment in a Phase III trial for ataluren in cystic fibrosis (nmCF).

- **We do not believe that a recommendation against conditional approval for ataluren is indicative of the CHMP's doubts regarding efficacy of ataluren.** Our checks suggest the CHMP has primarily been concerned with PTCT's ability to enroll an ongoing confirmatory Phase III trial should conditional approval be granted. We note that historically, despite strong underlying science and rational, subset analysis of results for drugs with new mechanisms of action have not met a *regulatory definition* for positive risk-benefit. We anticipate the confirmatory Phase III trial will read out in 2015 with a 2016 commercial launch in the U.S. and EU. We also see a potential for named patient sales occurring as early as late 2014 (see page 3).

- **New indications for ataluren's clinical development are to be selected in 2014 and may offer rapid routes to registration supported by excellent safety and tolerability from the current 600-patient strong clinical data set.** PTCT previously highlighted work in MPS disorders where small elevations in enzymes, which are not normally produced in the disease state, could result in significant and readily identifiable clinical benefit. We highlight that in rare lysosomal storage diseases, surrogate markers of efficacy may support accelerated approval. Furthermore, recently researchers at UBC discussed ataluren's efficacy in animal models of aniridia, a rare, non-sense mutation based condition that severely limits the vision of ~5,000 patients the U.S.

- **Recall that ataluren may be broadly applicable, beyond initial indications (DMD and CF), to 12% of all hereditary diseases and non-sense mutation-driven cancers.** There are ~2,400 genetically-defined rare diseases caused by non-sense mutations. We estimate that ataluren could be worth >$1.5 billion in peak sales in nmDMD and nmCF alone.

- **PTC/Roche's oral SMA compound recently entered clinical development and PTC is eligible for up to double-digit royalties on net sales.** The candidate demonstrated 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 showed that early therapy (day-0) with its compound appears to result in optimal restoration of near-normal life span and phenotypic characteristics. Demonstration of systemic impact of its SMA candidate, imparted by excellent bioavailability, included prevention of tail necrosis, as well as reduction in paw edema in SMA Type III mice. The candidate has also been shown to confer protection from neuromuscular junction denervation.

- **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.

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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 non-sense mutations. Ataluren may also be effective in treating ~2500 other rare diseases and certain cancers caused by non-sense 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 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. PTC is appealing a negative opinion from the EMA regarding conditional approval for ataluren for nmDMD in the EU and may here back in Q2:14, conditional approval is an unlikely scenario, in our opinion, but could result in a 2014 launch and 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 late 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. 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

~~Q4/Q1:14~~	~~CHMP SAG meeting regarding potential conditional approval for ataluren for nmDMD in the EU~~
~~Jan. 20-24~~	~~Potential conditional approval of ataluren for nmDMD in the EU~~
Q1:14	Initiation of a Phase III trial of ataluren in nmCF
Q1:14	MAA filing for conditional approval of ataluren for nmCF in the EU
~~H1:14~~	~~Initiation of Phase I/II trials of SMN2 candidate for SMA~~
Q2:14	Potential new opinion following a re-examination of the negative opinion regarding conditional approval of ataluren for nmDMD in the EU
Mid:14	Full enrollment in the confirmatory Phase III trial of ataluren in nmDMD
2014	Open label trial updates for ataluren as nmDMD at a scientific conference (US study safety only, EU efficacy at 0, 6,12,18 months)
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
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	Data from initial trials of PTCT's and Roche's SMA candidate
Late 2015	Potential accelerated approval of candidate for SMA
H1:16	FDA and MAA filing for full approval of ataluren for nmCF

The EU Conditional Approval Criteria

In order to make a recommendation for conditional approval the CHMP/EMA is required to ensure several key criteria are met. We delineate those below based on our checks of how the EMA views ataluren's ability to meet the criteria:

1) the risk-benefit balance of the medicinal product is positive;

> We believe ataluren's safety and tolerability is high as demonstrated in more than 600 patients. Given the existence of no other therapies (beyond steroids) for DMD, we believe the risk-benefit balance favors ataluren.

2) it is likely that the applicant will be in a position to provide the comprehensive clinical data;

> Ataluren's Phase III trial in patients with nmDMD is still enrolling (though our checks suggest nearly complete). An approved product could impact enrollment but also patients commitment to remain on study (ie: 50/50 chance of getting placebo in the study compared to a higher chance if approved product exists.) There are also a limited number of nmDMD patients available for the 220 patient trial. As of Dec 12, some sites in the EU have not yet begun recruiting patients.

3) unmet medical needs will likely be fulfilled;

> Given the existence of no other therapies (beyond steroids) for DMD we believe there is significant unmet medical need.

4) we believe the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required.

> Given the progression of existence of no other therapies (beyond steroids) for DMD we believe there is significant unmet medical need.

Named Patient Sales – Pre-Approval Selling that Could Prep for an Official Launch

Under a named-patient sales program, a pharmaceutical company may sell a drug to patients prior to approval. Generally, companies can charge "U.S." pricing for named-patient sales (Figure 1). The most recent and successful example of this program in our universe has been Gentium SpA's (GENT:NEUTRAL) program for defibrotide. Prior to approval in 2013, defibrotide generated annualized sales of $30 million on a named-patient basis penetrating roughly 15% of an estimated addressable market

Named-patient sales in the EU are regulated by individual countries and pricing ability and requirements vary. Named-patient sales are an important way to generate interest in the drug and connect with patients and advocacy organizations, but revenues are not likely to be meaningful until the drug is approved for reimbursement.

Figure 1: Named-Patient Sales Requirements/Pricing and Reporting

Country	Requirements	Drug Pricing	Payer	PTCT DMD Trial Sites	Reporting
Germany	'late approval stage' of EMA, Clinical trial initiated	Free pricing	National Health Insurance	2	Final report + AE's reported to BfArM or PEI
France	MA abroad	Free pricing	National Social Security system	4	Periodic report + AE's reported to ANSM
United Kingdom	"Special need"	Free Pricing	NHS Business Services Authority	2	AE's reported to MHRA
Italy	Orphan drugs in 'late stage of approval' by EMA	Negotiated	AIFA	3	No specific rules
Spain	Clinical trial	Negotiated	NHS	2	Efficacy and AE's must be reported to AEMPS
Brazil	Phase III initiated	None		2	AE's reported to ANVISA.
Turkey	Phase II complete	None		1	Ae's reported to Ministry of Health of Turkey.
Austria	Phase III trial completed	Negotiated	Regional Sickness funds	0	AE's reported to BASG
Belgium	Application for MA submitted, Phase III trial completed	None		1	No specific rules
Denmark		Free pricing	Hospital budget	0	AE's reported to DKMA
Norway	Approval in EEA, US or PCI/S country	Free pricing	MoHA or Hospital	0	No specific rules
Poland	Case by case	Negotiated	NHF	1	Not reported
Portugal	Case by case	Free pricing	Hospital budget	0	No specific rules
Sweden	EEA, US or PIC/s	Negotiated	County Council reimbursed by National Payer	2	AE's reported to MPA
Switzerland	"Equivalent approval program"	Free pricing	Swissmedic	1	Final report + AE's reported to Swissmedic

Source: Company data, Wedbush Securities, Inc.

Covered Companies Mentioned

Company Name	Ticker	Rating	Fair Value	Recent Price
Gentium SpA	GENT	Neutral	$57	$56.95

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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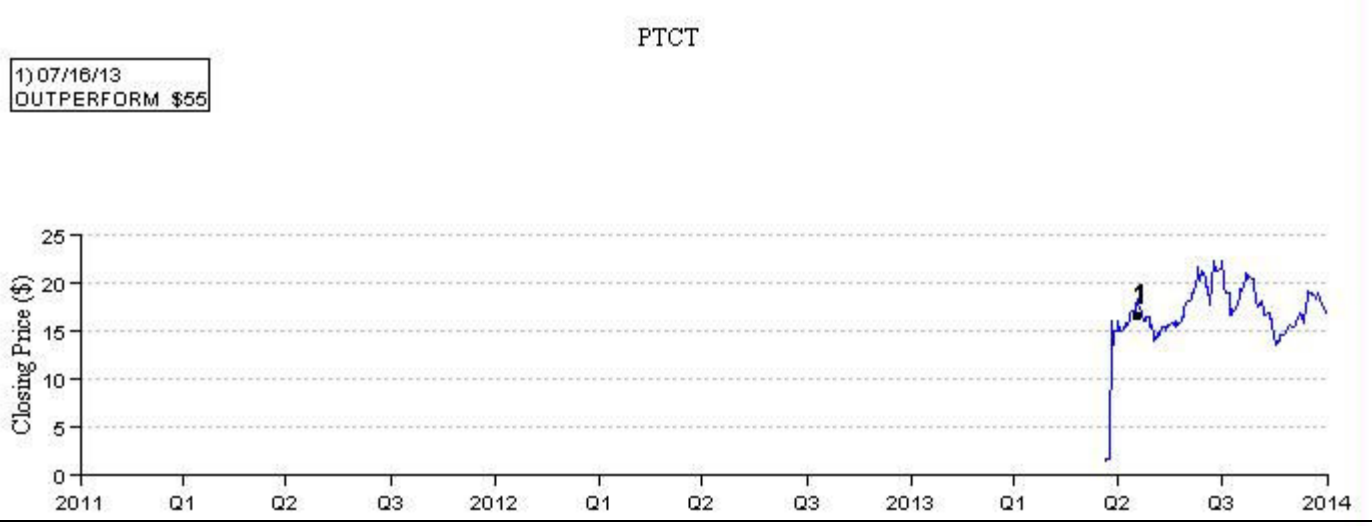

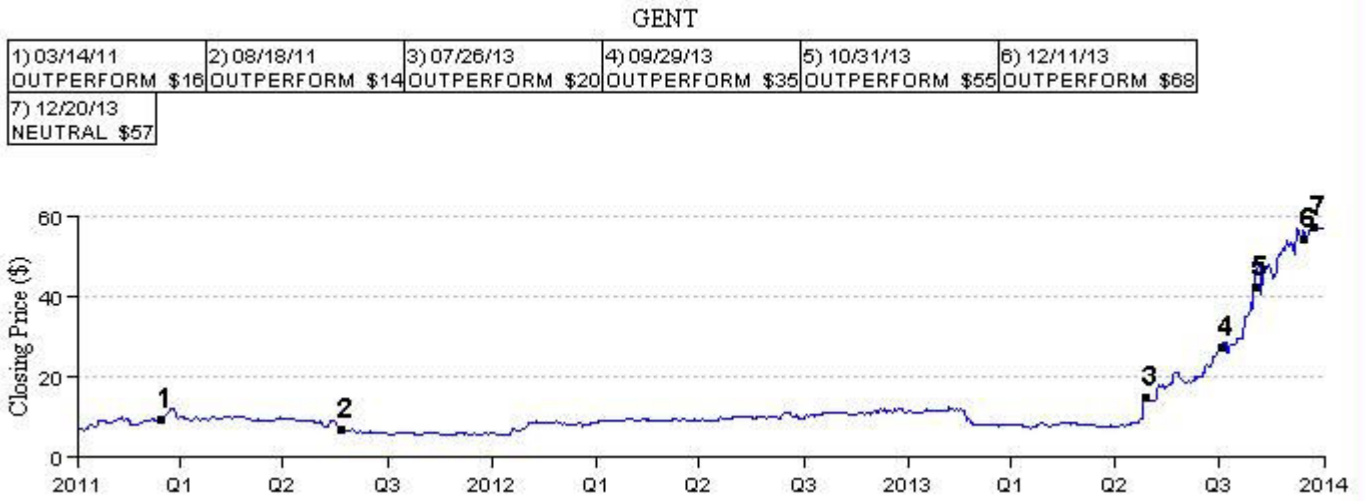

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