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Christopher N. Marai, Ph.D. (415) 274-6861

Gregory R. Wade, Ph.D. (415) 274-6863

David M. Nierengarten, Ph.D. (415) 274-6862

PTC Therapeutics (PTCT - OUTPERFORM): 2014 Update - SMA into the Clinic and New Indications for Ataluren Offer Significant Catalysts in the Year Ahead - Reiterate OUTPERFORM

Price: \$28.18

12-Month Price Target: \$55

- **We anticipate that the EMA/CHMP will announce their opinion regarding the conditional approval of ataluren in nmDMD next week at the January 20-23 meetings (result January 24).** Our checks suggest the CHMP is most concerned with the feasibility and impact of enrolling a Phase III trial should ataluren be conditionally approved. We believe that, despite recent strength, the market has assigned little chance of a conditional approval of ataluren in the EU and that progress in SMA and new indications for ataluren offer attractive upside in 2014.
- **Conditional approval of ataluren remains upside to our \$55 price target. We recommend aggressively accumulating shares of PTCT on any weakness or higher volume around this event in anticipation of several other catalysts for PTCT in 2014.** Importantly, we believe that the company may launch ataluren on a *named-patient* basis in the EU in late 2014.
- **PTCT announced that they anticipate initiation of a Phase I study in SMA in H1:14; additional data is expected in 2015.** We believe that as an orally bioavailable small molecule PTCT/Roche's (ROG: not covered) SMA candidate is differentiated from the ISIS/BioGen Idec (ISIS: not covered, BIIB: not covered) approach for SMA. We highlight that ease and safety of systemic delivery of PTCT's compound 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.
- **PTC/Roche's oral SMA compound 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 their compound appears to result in optimal restoration of near-normal life span and phenotypic characteristics. Demonstration of systemic impact of their 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.
- **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.
- **We view PTCT with an EV of ~\$440 and breadth of their clinical programs as undervalued relative to peers at similar stages of development.** We anticipate that the next catalysts for PTCT shares will include initiation of a Phase I SMA, 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 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.

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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 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 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. Additionally, PTC may also receive conditional approval for ataluren for nmDMD in the EU by Q1:14, a scenario which would 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 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 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
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

Analyst Certification

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Company	Disclosure
PTC Therapeutics	1,3,4,5

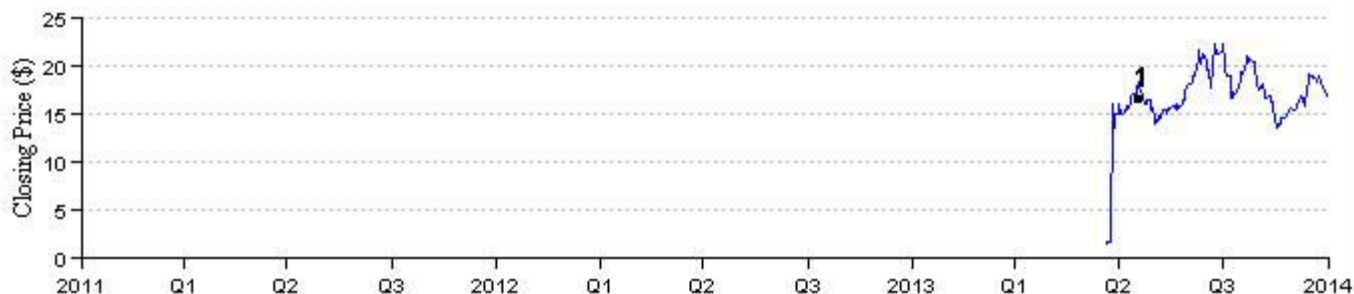
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PTCT

1) 07/16/13
OUTPERFORM \$55


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

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RESEARCH DEPT. * (213) 688-4505 * www.wedbush.com

EQUITY TRADING Los Angeles (213) 688-4470 / (800) 421-0178 * EQUITY SALES Los Angeles (800) 444-8076

CORPORATE HEADQUARTERS (213) 688-8000

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WEDBUSH

EQUITY RESEARCH DEPARTMENT

(213) 688-4529

DIRECTOR OF RESEARCH

Mark D. Benson (213) 688-4435

MANAGER, RESEARCH OPERATIONS

Ellen Kang (213) 688-4529

RETAIL AND CONSUMER

Consumer Products

Rommel T. Dionisio (212) 938-9934
Kurt M. Frederick, CFA CPA (415) 274-6822
Alicia Reese (212) 938-9927

Footwear, Apparel and Accessories

Corinna Freedman (212) 668-9876

Healthy Lifestyles

Kurt M. Frederick, CFA CPA (415) 274-6822

Restaurants

Nick Setyan (213) 688-4519
Colin Radke (213) 688-6624

Specialty Retail: Hardlines

Joan L. Storms, CFA (213) 688-4537
John Garrett, CFA (213) 688-4523

Seth Basham, CFA (212) 938-9954

RETAIL/CONSUMER MARKET RESEARCH

Gabriella Santaniello (213) 688-4557

INDUSTRIAL GROWTH TECHNOLOGY

Clean Technology

Craig Irwin (212) 938-9926
Min Xu (212) 938-9925

Environmental Services / Building Products

Al Kaschalk (213) 688-4539
Taryn Kuida (213) 688-4505

Water and Renewable Energy Solutions

David Rose, CFA (213) 688-4319
James Kim (213) 688-4380

TECHNOLOGY, INTERNET, MEDIA & SOCIAL MEDIA

Communications and Application Software

Shyam Patil, CFA (213) 688-8062
Andy Cheng (213) 688-4548

Communications Equipment

Rohit Chopra (212) 668-9871
Sanjit Singh (212) 938-9922
Ryan Flanagan (212) 938-9942

Computer Services: Financial Technology

Gil B. Luria (213) 688-4501
Aaron Turner (213) 688-4429

Enterprise Software

Steve Koenig (415) 274-6801
Kevin Ikeda (213) 688-4423

Entertainment: Retail

Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

Entertainment: Software

Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

Internet: Media and Gaming

Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

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Shyam Patil, CFA (213) 688-8062
Andy Cheng (213) 688-4548

Media

James Dix, CFA (213) 688-4315

Movies and Entertainment

Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

Semiconductors

Betsy Van Hees (415) 274-6869
Ryan Jue, CFA (415) 263-6669

LIFE SCIENCES AND HEALTH CARE

Biotechnology/Biopharmaceuticals/BioDefense

Gregory R. Wade, Ph.D. (415) 274-6863
David M. Nierengarten, Ph.D. (415) 274-6862
Christopher N. Marai, Ph.D. (415) 274-6861

Emerging Pharmaceuticals

Liana Moussatos, Ph.D. (415) 263-6626
Richard Lau, CFA (415) 274-6851

Healthcare Services - Managed Care

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San Francisco (415) 274-6811
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CORPORATE HEADQUARTERS

1000 Wilshire Blvd., Los Angeles, CA 90017-2465

Tel: (213) 688-8000 www.wedbush.com