

PTC Therapeutics (PTCT)

Q3 Update - 2014 a Value-Creating Year for PTCT - SMA Program Updates and Next Indication for Ataluren - Reiterate OUTPERFORM

- PTCT reported their Q3:13 results and recently provided an update on their extensive pre-clinical programs at their analyst day. PTCT ended Q3:13 with \$175M in cash, which we anticipate is sufficient to support operations into H2:15.
- PTCT announced on the call that the CHMP will now convene a scientific advisory group (SAG) as part of the conditional approval process of ataluren for nmDMD. The meeting may shift the final opinion from the CHMP decision into Q1:14, a potential positive as our checks suggest the CHMP is most concerned with the feasibility of enrolling a Phase III trial should ataluren be conditionally approved. Full enrollment in the Phase III trial of ataluren in nmDMD is expected by mid-2014. We also anticipate that the scientific advisory group, which will be made up of DMD experts and patient advocates in the EU, is likely to be positively disposed to ataluren's risk/benefit profile. Conditional approval of ataluren for nmDMD in the EU remains upside to our estimates and \$55 price target.
- SMA program updates in 2014 are likely to highlight clinical and regulatory paths forward for the oral Roche/PTC SMA candidate. We believe that as an orally bioavailable small molecule, PTCT/Roche's SMA candidate is differentiated from the ISIS (ISIS:Not Covered) and PMO approaches 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.
- Anticipated near-term progress in PTCT's pipeline that could drive valuation include, selection of ataluren's next indication, and the start of potentially registration-worthy Phase I/II studies for PTCT's SMA candidate that is partnered with Roche. We view PTCT with an enterprise value of <\$300M as undervalued relative to companies at similar stages of development.
- 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.

FYE Dec	2012A	2013E			2014E		
REV	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	--	\$7.1A		N/A	\$3.6E		\$3.5E
Q2 Jun	--	6.9A		6.9A	7.5E		\$3.5E
Q3 Sep	--	16.3A	17.0A	12.6A	7.5E		\$3.5E
Q4 Dec	--	6.1E		7.5E	\$7.5E		\$3.5E
Year*	--	\$36.4E	\$37.1E	\$34.1E	\$26.1E		\$3.5E
Change	--	--			--		
EPS	2012A	2013E			2014E		
	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	--	(\$3,244)A		N/A	(\$0.65)E	(\$0.62)E	(\$0.67)E
Q2 Jun	--	(5.51)A		(5.51)A	(\$0.51)E	(\$0.48)E	(\$0.67)E
Q3 Sep	--	(0.19)A	(0.02)A	(0.30)A	(\$0.53)E	(\$0.50)E	(\$0.67)E
Q4 Dec	--	(0.50)E	(0.48)E	(0.52)E	(\$0.55)E	(\$0.53)E	(\$0.67)E
Year*	--	(\$3.63)E	(\$3.18)E	(\$3.57)E	(\$0.55)E	(\$0.53)E	(\$0.67)E
P/E	--	--			--		
Change	--	--			--		

Consensus estimates are from Thomson First Call.

* Numbers may not add up due to rounding.

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Price
\$16.17

Rating
OUTPERFORM

12-Month Price Target
\$55

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Company Information

Shares Outst (M)	23.8
Market Cap (M)	\$376.1
52-Wk Range	\$13.04 - \$24.38
Book Value/sh	\$14.75
Cash/sh	\$6.61
Enterprise Value (M)	\$218.9
LT Debt/Cap %	0.0
Cash Burn (M)	\$45.6
Current Cash (M)	\$157.2

Company Description

PTC Therapeutics is a biopharmaceutical company focused on the development of orally administered, proprietary, small molecule drugs that target post-transcriptional control processes for orphan and ultra-orphan disorders including DMD and CF.



Source: Thomson Reuters

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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 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 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 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 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

H2:13	Preparations for early access programs (in select territories) for ataluren in nmDMD
Dec. 17, 2013	Lockup expiration
Dec. 20	Potential conditional approval of ataluren for nmDMD in the EU
Q4/Q1:14	CHMP SAG meeting regarding potential conditional approval for ataluren for nmDMD in the EU
Jan. 24	Potential conditional approval of ataluren for nmDMD in the EU
Feb. 21	Potential conditional approval of ataluren for nmDMD in the EU
Mar. 21	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
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
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

Financial Model



Christopher N. Marai Ph.D.

11/14/2013

PTC Therapeutics, Inc.

Annual Financial Results & Projections

(\$ in thousands except per share data)

Ticker: PTCT (Nasdaq)

	FY:12A	Q1:13	Q2:13	Q3:13	Q4:13	FY:13E	FY:14E	FY:15E	FY:16E	FY:17E	FY:18E	FY:19E
Revenue:												
Ataluren Sales US - DMD	0	0	0	0	0	0	0	0	49,618	216,553	313,599	335,577
Ataluren Sales EU - DMD	0	0	0	0	0	0	0	0	35,543	185,637	310,494	345,451
Ataluren Sales ROW - DMD	0	0	0	0	0	0	0	0	715	10,692	47,266	93,713
Ataluren Sales US - CF	0	0	0	0	0	0	0	0	0	94,984	267,911	344,683
Ataluren Sales EU - CF	0	0	0	0	0	0	0	0	0	37,354	185,142	367,238
Ataluren Sales ROW - CF	0	0	0	0	0	0	0	0	0	0	1,010	12,187
Grant and other revenues	5,167	1,070	986	834	1,100	3,990	5,600	4,000	0	0	0	0
Collaboration revenue	28,779	6,072	5,868	15,455	5,000	32,395	20,500	18,000	1,000	1,000	1,000	1,000
Total Revenues	\$33,946	\$7,142	\$6,854	\$16,289	\$6,100	\$36,385	\$26,100	\$22,000	\$86,876	\$546,221	\$1,126,423	\$1,499,849
Cost and Expenses:												
Costs of goods sold	0	0	0	0	0	0	0	0	8,588	54,522	112,542	149,885
Research and Development	46,139	11,257	14,712	13,886	12,000	51,855	51,000	57,000	62,305	66,497	67,837	69,204
Sales, General and Administrative	14,615	4,461	6,595	6,679	6,500	24,235	30,000	31,500	43,000	52,000	53,313	55,478
Other	0	0	0	0	0	0	0	0	0	0	0	0
Total Costs and Expenses	\$60,754	\$15,718	\$21,307	\$20,565	\$18,500	\$76,090	\$81,000	\$88,500	\$113,893	\$173,019	\$233,692	\$274,566
Operating income (loss)	(26,808)	(8,576)	(14,453)	(4,276)	(12,400)	(39,705)	(54,900)	(66,500)	(27,017)	373,202	892,731	1,225,283
Net Interest Income (Expense)	(1,210)	(6,162)	(114)	27	472	(5,777)	1,293	866	1,549	2,795	8,719	18,128
Other income / (Expense)	1,783	53	(19)	(167)	0	(133)	0	0	0	0	0	0
Income Before Income Taxes	(26,235)	(14,685)	(14,587)	(4,416)	(11,928)	(45,616)	(53,607)	(65,634)	(25,468)	375,997	901,449	1,243,411
Net Income	\$133,341	(\$14,685)	(\$14,587)	(\$4,416)	(\$11,928)	(\$45,616)	(\$53,607)	(\$65,634)	(\$25,468)	\$350,396	\$707,254	\$932,558
GAAP Net Income	\$133,341	(\$29,543)	(\$14,587)	(\$4,416)	(\$11,928)	(\$60,474)	(\$53,607)	(\$65,634)	(\$25,468)	\$340,396	\$697,254	\$922,558
GAAP Basic EPS with sFAS123	-	(3244.59)	(5.51)	(0.19)	(0.50)	(3.63)	(2.24)	(2.63)	(0.98)	13.38	26.90	35.34
GAAP Diluted EPS with sFAS123	-	(3244.59)	(5.51)	(0.19)	(0.50)	(3.63)	(2.24)	(2.63)	(0.98)	13.38	26.90	35.34
Weighted shares outstanding	3	5	2,649	23,803	23,828	12,571	23,853	24,991	26,091	26,191	26,291	26,391
Fully diluted shares outstanding	17	5	2,649	23,803	23,828	12,571	23,891	24,991	26,091	26,191	26,291	26,391
Cash Burn	(26,808)	(14,685)	(14,587)	(4,416)	(11,928)	(45,616)	(53,607)	(65,634)	(25,468)	-	-	-
Cash Balance	2,726	50,247	165,678	157,227	129,687	129,687	74,654	92,940	152,405	486,926	1,179,394	2,104,664

Source: Wedbush Securities and PacGrow Life Sciences

Analyst Biography

Chris Marai is an Analyst covering the Biotechnology/Biopharmaceuticals/BioDefense sector. Prior to Wedbush PacGrow Life Sciences, Dr. Marai was at Morgan Stanley where he specialized in quantitative modeling; he has also consulted for structure-based drug design companies and biotechnology startups.

Dr. Marai holds a B.S. in Chemistry from Trinity College, University of Toronto and a Ph.D. in Biochemistry and Structural Biology from Stony Brook University, New York.

Christopher's Edge: Dr. Marai has a strong quantitative background and has covered a wide range of disease areas including gastrointestinal, CNS, oncology and rare diseases. His quantitative background has translated into an exceptional track record predicting binary events and assessing risk as a sell-side analyst.

Analyst Certification

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Company	Disclosure
PTC Therapeutics	1,3,5

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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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