

## PTC Therapeutics

### Our Thoughts Post the 3Q Call

PTC reported a GAAP loss per share of \$0.19. The most notable update on the call was related to conditional approval of ataluren in Europe for Duchene muscular dystrophy (DMD). PTC indicated the CHMP is now expected to convene a Scientific Advisory Group (SAG) to provide a robust discussion with input from experts and patient advocacy groups. A final decision is now expected in 1Q14 (previously 4Q13), but we continue to believe conditional approval is unlikely. Importantly, timelines for ataluren in nmDMD remain on track with completion of enrollment in phase 3 expected in mid-2014 and data in mid-2015. We remain confident in the phase 3 enrollment criteria and study design which provides for a high probability of success, in our view. Given the continued progress and our expectations, we reiterate our Overweight rating.

- Ataluren phase 3 program.** The ongoing phase 3 trial in nmDMD is on track with completion of enrollment expected in mid-2014 and data in mid-2015. PTC reiterated it remains confident in the phase 3 trial design. Importantly, throughout the development process, feedback from the FDA has been consistent, indicating dystrophin is not a reliable biomarker and 6MWD is an acceptable endpoint in an appropriately powered study. Of note, this is also consistent with the FDA's message to Sarepta earlier this week. Additionally, PTC remains on track to begin a phase 3 trial of ataluren in nmCF in 1H14.
- CHMP conditional approval update.** Ataluren is currently under review for conditional nmDMD approval in Europe. PTC indicated the CHMP now expects to convene a SAG that will involve experts and patient advocacy groups. While this will allow for a more robust discussion of ataluren in nmDMD, PTC characterized the potential for approval as "uncertain." We continue to view conditional approval as a low probability event. Given this new development, a decision from the CHMP is now expected in 1Q14 from prior expectations for 4Q13. Additionally, PTC intends to delay plans for an MAA filing for condition approval of ataluren in nmCF until 1Q14 (previously YE13) to focus on the regulatory process for nmDMD.
- Adjusting estimates.** Based on 3Q results, we are making adjustments to our model. Our 2013-2015 GAAP EPS estimates change to -\$2.94, -\$2.72, and -\$3.12, respectively, from -\$3.41, -\$2.68, and -\$3.09.
- Reiterate Overweight rating.**

#### PTC Therapeutics (PTCT;PTCT US)

FYE Dec	2011A	2012A	2013E	2013E	2014E	2014E	2015E	2015E
			(Prev)	(Curr)	(Prev)	(Curr)	(Prev)	(Curr)
EPS Reported (\$)								
Q1 (Mar)	-	-	(1.51)A	(1.51)A	-	-	-	-
Q2 (Jun)	-	-	(0.59)A	(0.59)A	-	-	-	-
Q3 (Sep)	-	-	(0.64)	(0.19)A	-	-	-	-
Q4 (Dec)	-	-	(0.68)	(0.66)	-	-	-	-
FY	5.39	7.75	(3.41)	(2.94)	(2.68)	(2.72)	(3.09)	(3.12)

Source: Company data, Bloomberg, J.P. Morgan estimates.

## Overweight

PTCT, PTCT US

Price: \$16.31

Price Target: \$26.00

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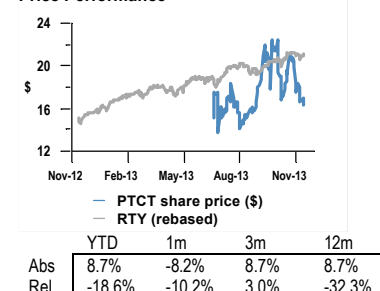
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#### Price Performance



#### Company Data

Price (\$)	16.31
Date Of Price	13 Nov 13
52-week Range (\$)	24.38-13.04
Market Cap (\$ mn)	445.32
Fiscal Year End	Dec
Shares O/S (mn)	27
Price Target (\$)	26.00
Price Target End Date	30-Dec-14

#### See page 4 for analyst certification and important disclosures.

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## Changes to Our Model

Based on 3Q results, we are making adjustments to our model. Our 2013-2015 GAAP EPS estimates change to -\$2.94, -\$2.72, and -\$3.12, respectively, from -\$3.41, -\$2.68, and -\$3.09.

Table 1: Changes to Our Model

	2013		2014		2015	
	Old	New	Old	New	Old	New
Total Revenues	26.0	36.3	10.0	10.0	7.0	7.0
Cost of sales	0.0	0.0	0.0	0.0	0.0	0.0
Research and development	55.0	54.4	60.5	59.8	66.5	65.8
Sales, general and administrative	26.1	24.7	27.4	26.0	32.8	31.2
Operating Income	-55.0	-42.8	-77.8	-75.8	-92.3	-89.9
Net Income	-56.4	-43.6	-76.3	-74.3	-90.8	-88.4
Diluted GAAP EPS	-3.41	-2.94	-2.68	-2.72	-3.09	-3.12

AtaSource: J.P. Morgan estimates.

## Investment Thesis

PTC is focused on therapies for orphan genetic diseases, with the most advanced compound being ataluren, currently in phase 3 development for Duchene muscular dystrophy (DMD; data 1H15) and cystic fibrosis (CF; beginning second study 1H14). We believe insights gained in prior ataluren clinical studies maximize the probability for success in phase 3. While ataluren, which is unpartnered, has potential in both indications, we conservatively include revenues only for DMD with peak WW sales of \$700M, which could easily double should CF also be successful. Given a high probability of success for ataluren in DMD, good optionality in CF, plus a “free call option” on EU approval in DMD in 4Q13, we rate PTCT shares Overweight.

## Valuation

Our \$26 price target for PTCT is based on our sum-of-the-parts analysis including only ataluren in nmDMD. We project ataluren nmDMD sales to 2024, consistent with IP protection, assume no terminal value and an 8% discount rate. We further assume an 80% probability of success for ataluren in Phase 3. We believe this appropriately reflects the risks of the phase 3 DMD program. We derive a value of \$24/share for ataluren. This, taken in combination with net cash of \$2/share, supports our December 2014 PT of \$26.

## Risks to Rating and Price Target

Risks to our Overweight rating include: 1) ataluren's inability to demonstrate a meaningful benefit in DMD and CF in late stage clinical trials, 2) regulatory risk in both the US and EU, 3) failure for ataluren to gain meaningful market shares if approved, and 4) potential future share dilution.

## PTC Therapeutics: Summary of Financials

Income Statement - Annual	FY12A	FY13E	FY14E	FY15E	Income Statement - Quarterly	1Q13A	2Q13A	3Q13A	4Q13E
Revenues	34	36	10	7	Revenues	7A	7A	16A	6
Cost of products sold	0	0	0	0	Cost of products sold	0A	0A	0A	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(15)	(25)	(26)	(31)	SG&A	(4)A	(7)A	(7)A	(7)
R&D	(46)	(54)	(60)	(66)	R&D	(11)A	(15)A	(14)A	(15)
Operating income	(27)	(43)	(76)	(90)	Operating income	(9)A	(14)A	(4)A	(16)
EBITDA	(27)	(43)	(76)	(90)	EBITDA	(9)A	(14)A	(4)A	(16)
Net interest (income) / expense	(1)	(1)	1	1	Net interest (income) / expense	(1)A	(0)A	(0)A	(0)
Other income / (expense)	2	(0)	1	1	Other income / (expense)	0A	(0)A	(0)A	0
Income taxes	0	0	0	0	Income taxes	0A	0A	0A	0
Net income - GAAP	(26)	(44)	(74)	(88)	Net income - GAAP	(9)A	(15)A	(4)A	(16)
Net income - recurring	133	(58)	(74)	(88)	Net income - recurring	(24)A	(15)A	(4)A	(16)
Diluted shares outstanding	17	20	27	28	Diluted shares outstanding	16A	25A	24A	24
EPS - excluding non-recurring	(1.52)	(2.19)	(2.72)	(3.12)	EPS - excluding non-recurring	(0.57)A	(0.59)A	(0.19)A	(0.66)
EPS - recurring	7.75	(2.94)	(2.72)	(3.12)	EPS - recurring	(1.51)A	(0.59)A	(0.19)A	(0.66)
Balance Sheet and Cash Flow Data	FY12A	FY13E	FY14E	FY15E	Ratio Analysis	FY12A	FY13E	FY14E	FY15E
Cash and cash equivalents	3	145	152	64	Sales growth	(67.8%)	6.9%	(72.4%)	(30.0%)
Accounts receivable	-	-	-	-	EBIT growth	(187.7%)	59.7%	77.0%	18.7%
Inventories	-	-	-	-	EPS growth - recurring	43.7%	(137.9%)	(7.4%)	14.9%
Other current assets	2	2	2	2	Gross margin	-	-	-	-
Current assets	5	147	154	65	EBIT margin	(79.0%)	(118.0%)	(757.6%)	(1284.8%)
PP&E	8	9	9	9	EBITDA margin	(79.0%)	(118.0%)	(757.6%)	(1284.8%)
Total assets	13	156	163	75	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	5	5	5	5	Net margin	392.8%	(161.2%)	(742.6%)	(1263.4%)
Total liabilities	32	32	32	32	Net Debt / EBITDA	(8.0%)	328.5%	194.6%	65.3%
Shareholders' equity	(19)	124	131	43	Net Debt / Capital (book)	(12.9%)	859.6%	918.1%	372.4%
Net income (including charges)	(26)	(58)	(74)	(88)	Return on assets (ROA)	466.1%	(69.1%)	(46.5%)	(74.3%)
D&A	3	3	3	3	Return on equity (ROE)	(619.6%)	(110.9%)	(58.1%)	(101.5%)
Change in working capital	(25)	0	0	0	Enterprise value / sales	-	-	-	-
Other	1	0	0	0	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	(48)	(56)	(72)	(86)	Free cash flow yield	(16.7%)	(17.9%)	(16.5%)	(19.0%)
Capex	(0)	(3)	(3)	(3)					
Free cash flow	(47)	(58)	(74)	(88)					
Cash flow from investing activities	(0)	(3)	(3)	(3)					
Cash flow from financing activities	22	114	81	0					
Dividends	-	-	-	-					
Dividend yield	-	-	-	-					

Source: Company reports and J.P. Morgan estimates.

Note: \$ in millions (except per-share data). Fiscal year ends Dec

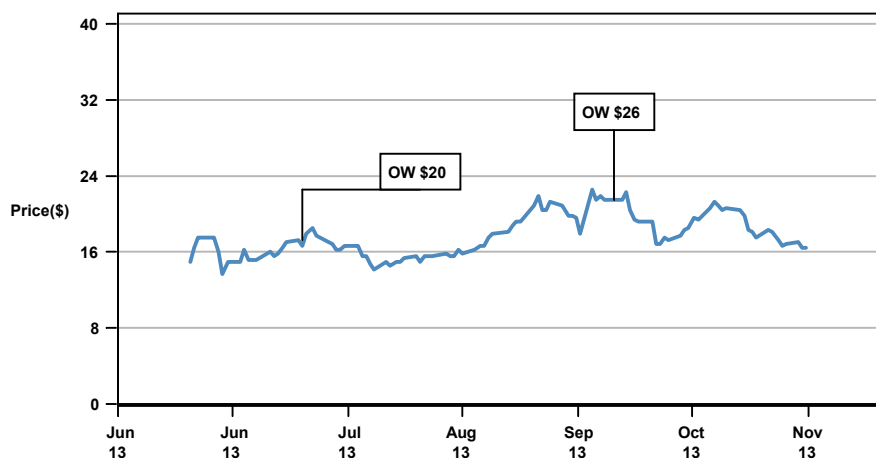
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PTC Therapeutics (PTCT, PTCT US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	17.17	20.00
27-Sep-13	OW	21.45	26.00

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.  
Initiated coverage Jul 15, 2013.

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