

## PTC Therapeutics

### Our Thoughts Post the 4Q13 Call

PTC reported a GAAP loss per share of \$0.75 (cons: -\$0.58). Importantly, the company is making very good progress with the pipeline with the phase 3 trial of ataluren in DMD on track to complete enrollment in mid-2014 (data in mid-2015e). In our view, the phase 3 design provides for a high probability of success with what has been careful patient selection thus far. Additionally, a confirmatory phase 3 trial of ataluren in CF is expected to begin in 1H14. Finally, a proof-of-concept study of ataluren targeting a nonsense mutation disease (undisclosed) is expected to begin in 2014. Indeed, PTC noted that ataluren is active in many indications (>20 publications) and could move forward in multiple studies in 2015. Of note, a final decision on conditional approval of ataluren in nmDMD in Europe is expected in 2Q14, though we view conditional approval as very unlikely event. Given the continued progress and our nmDMD expectations, we reiterate our Overweight rating. Additionally, we are raising our YE14 PT to \$40 from \$26 and this now includes value for the pipeline/platform technology, most notably SMA.

- **Ataluren phase 3 program:** The ongoing phase 3 trial of ataluren in nmDMD remains on track to complete enrollment in mid-2014 with data expected in mid-2015. We believe insights gained during the prior phase 2b study, which were applied to the ongoing phase 3 trial, optimize the responsive patient population, raising the probability of success. We project peak WW sales of ataluren in nmDMD of ~\$700M.
- **Pipeline – other:** Ataluren in nmCF – Enrollment in the phase 3 nmCF trial remains on track to begin in 1H14 with data likely in mid-2016. PTC noted that the trial design will be largely similar to the prior phase 3 study (primary endpoint FEV1 with pulmonary exacerbations as a secondary endpoint), but importantly will exclude use of tobramycin. SMA program – Recall, this program is partnered with Roche and recently entered a phase 1 trial in January of this year.
- **2014 Guidance:** PTC guided to 2014 total operating expense of \$85-95M, excluding \$15M in stock-based compensation. Additionally, PTC expects to end 2014 with ~\$175-185M in cash, cash equivalents and marketable securities.
- **Adjusting estimates:** Based on 4Q results and recent trends, we are making adjustments to our model. We have fine tuned operating expenses and increase shares outstanding to reflect the recent public offering (5.2M shares or \$126.5M gross proceeds). Our 2014 and 2015 GAAP EPS estimates move down to -\$2.79 and -\$3.15, from -\$2.72 and -\$3.12, respectively.
- **Reiterate Overweight rating.**

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

FYE Dec	2011A	2012A	2013A	2014E (Prev)	2014E (Curr)	2015E (Prev)	2015E (Curr)
EPS Reported (\$)							
Q1 (Mar)	-	-	(1.51)	-	(0.44)	-	-
Q2 (Jun)	-	-	(0.59)	-	(0.72)	-	-
Q3 (Sep)	-	-	(0.19)	-	(0.78)	-	-
Q4 (Dec)	-	-	(0.75)	-	(0.82)	-	-
FY	5.39	7.75	(2.75)	(2.72)	(2.79)	(3.12)	(3.15)

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

## Overweight

PTCT, PTCT US

Price: \$33.38

▲ **Price Target: \$40.00**

Previous: \$26.00

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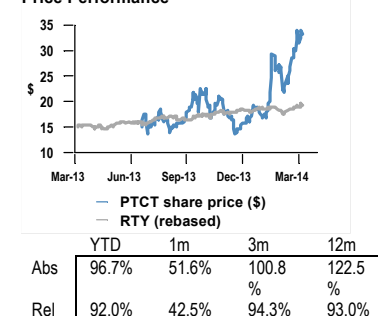
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#### Price Performance



#### Company Data

Price (\$)	33.38
Date Of Price	06 Mar 14
52-week Range (\$)	34.65-13.04
Market Cap (\$ mn)	940.58
Fiscal Year End	Dec
Shares O/S (mn)	28
Price Target (\$)	40.00
Price Target End Date	30-Dec-14

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## Changes to Our Model

Based on 4Q results and recent trends, we are making adjustments to our model. We have fine tuned operating expenses and increase shares outstanding to reflect the recent public offering (5.2M shares or \$126.5M gross proceeds). Our 2014 and 2015 GAAP EPS estimates decrease to -\$2.79 and -\$3.15, from -\$2.72 and -\$3.12, respectively.

Table 1: Changes to Our Model

	2013A		2014		2015	
	Old	New	Old	New	Old	New
Total Revenues	36.3	34.7	10.0	23.5	7.0	22.0
Cost of sales	0.0	0.0	0.0	0.0	0.0	0.0
Research and development	54.4	54.9	59.8	69.5	65.8	76.5
Sales, general and administrative	24.7	25.2	26.0	34.1	31.2	40.9
Operating Income	-42.8	-45.4	-75.8	-80.1	-89.9	-95.4
Net Income	-43.6	-45.9	-74.3	-78.6	-88.4	-93.9
Diluted GAAP EPS	-2.94	-2.75	-2.72	-2.79	-3.12	-3.15

Source: J.P. Morgan estimates.

## Investment Thesis, Valuation and Risks

### PTC Therapeutics (*Overweight; Price Target: \$40.00*)

#### 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 mid-2015) 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 1Q14, we rate PTCT shares Overweight.

#### Valuation

Our \$40 December 2014 price target for PTCT is based on our sum-of-the-parts analysis including ataluren only in nmDMD as well as the pipeline/platform technology. We project ataluren nmDMD sales to 2024, consistent with IP protection, assume no terminal value and an 8% discount rate. We further assume a high 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 \$25/share for ataluren. This, taken in combination with \$7/share for the pipeline/platform technology and net cash of \$7/share, supports our December 2014 PT of \$40.

#### 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	FY13A	FY14E	FY15E	FY16E	Income Statement - Quarterly	1Q14E	2Q14E	3Q14E	4Q14E
Revenues	35	24	22	35	Revenues	12	4	4	4
Cost of products sold	0	0	0	(2)	Cost of products sold	0	0	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(25)	(34)	(41)	(57)	SG&A	(8)	(8)	(9)	(9)
R&D	(55)	(70)	(76)	(84)	R&D	(16)	(17)	(18)	(19)
Operating income	(45)	(80)	(95)	(109)	Operating income	(12)	(21)	(23)	(24)
EBITDA	(45)	(80)	(95)	(109)	EBITDA	(12)	(21)	(23)	(24)
Net interest (income) / expense	(1)	1	1	2	Net interest (income) / expense	0	0	0	0
Other income / (expense)	0	0	1	1	Other income / (expense)	0	0	0	0
Income taxes	0	0	0	0	Income taxes	0	0	0	0
Net income - GAAP	(46)	(79)	(94)	(107)	Net income - GAAP	(12)	(21)	(22)	(24)
Net income - recurring	(61)	(79)	(94)	(107)	Net income - recurring	(12)	(21)	(22)	(24)
Diluted shares outstanding	22	28	30	32	Diluted shares outstanding	26	29	29	29
EPS - excluding non-recurring	(2.08)	(2.79)	(3.15)	(3.30)	EPS - excluding non-recurring	(0.44)	(0.72)	(0.78)	(0.82)
EPS - recurring	(2.75)	(2.79)	(3.15)	(3.30)	EPS - recurring	(0.44)	(0.72)	(0.78)	(0.82)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	143	175	80	78	Sales growth	2.2%	(32.3%)	(6.4%)	58.1%
Accounts receivable	-	-	-	-	EBIT growth	69.3%	76.4%	19.1%	14.4%
Inventories	-	-	-	-	EPS growth - recurring	(135.5%)	1.4%	12.9%	4.8%
Other current assets	2	2	2	2	Gross margin	-	-	-	-
Current assets	145	176	82	80	EBIT margin	(130.8%)	(340.8%)	(433.5%)	(313.6%)
PP&E	9	9	9	9	EBITDA margin	(130.8%)	(340.8%)	(433.5%)	(313.6%)
Total assets	154	185	92	90	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	5	5	5	5	Net margin	(175.1%)	(334.5%)	(426.6%)	(306.4%)
Total liabilities	32	32	32	32	Net Debt / EBITDA	304.7%	211.8%	79.2%	67.2%
Shareholders' equity	122	154	60	58	Net Debt / Capital (book)	845.6%	1056.6%	479.1%	473.9%
Net income (including charges)	(61)	(79)	(94)	(107)	Return on assets (ROA)	(72.8%)	(46.3%)	(67.7%)	(117.5%)
D&A	3	3	3	3	Return on equity (ROE)	(117.8%)	(57.1%)	(88.0%)	(181.3%)
Change in working capital	0	0	0	0	Enterprise value / sales	-	-	-	-
Other	0	0	0	0	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	(58)	(76)	(91)	(104)	Free cash flow yield	(8.2%)	(8.3%)	(9.4%)	(9.7%)
Capex	(3)	(3)	(3)	(3)					
Free cash flow	(61)	(78)	(93)	(105)					
Cash flow from investing activities	(3)	(3)	(3)	(3)					
Cash flow from financing activities	114	110	0	105					
Dividends	-	-	-	-					
Dividend yield	-	-	-	-					

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

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

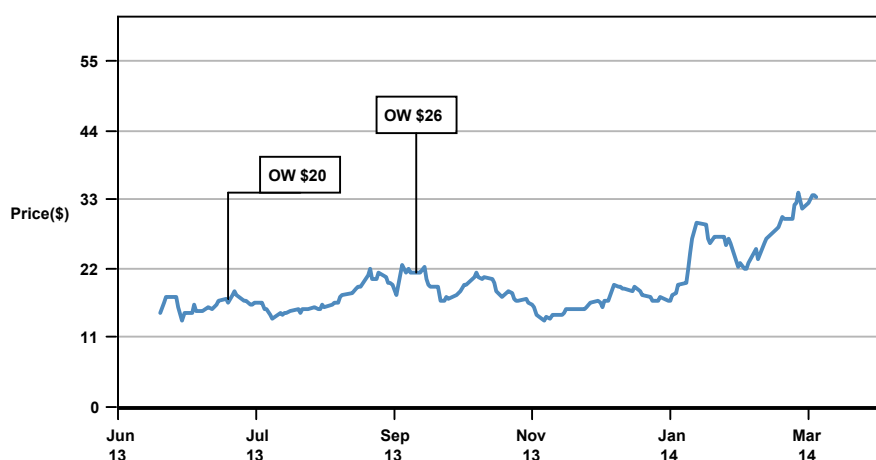
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Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.  
Initiated coverage Jul 15, 2013.

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