

PTC Therapeutics

Our Thoughts Post the 2Q Call

PTC reported a GAAP loss per share of \$0.59 (cons: -\$0.73; JPMe: -\$0.77). Overall, there were no major updates on the call, with PTC providing an overview of the company and ongoing development programs. Looking forward, we believe PTC is making solid/steady progress on key ataluren programs in Duchene muscular dystrophy (DMD) and cystic fibrosis (CF; see details below). Given insights from prior trials, we believe both programs now have a higher probability of success. As such, we are reiterating our Overweight rating on PTCT shares, based on the DMD potential alone (CF provides meaningful call option). We are establishing a December 2014 PT of \$20 (prior December 2013 PT of \$20).

- **Ataluren in nmDMD:** The phase 3 trial of ataluren in nmDMD is on track to complete enrollment in mid-2014, with data expected in mid-2015 (no real change in timelines). On the call, it was highlighted that all sites in the phase 2b trial are participating in the phase 3 study. Additionally, a response from the CHMP on conditional approval of ataluren in nmDMD is expected by YE13. We view conditional approval as low probability. We estimate the market potential of Ataluren in nmDMD to be \$700M.
- **Pipeline – other: Ataluren in nmCF** – Enrollment in the phase 3 nmCF trial is on track to begin in 1H14. We do not currently include the CF opportunity in our model. Of note, on the call, it was highlighted that PTC plans to file for conditional approval in the EU by YE13. Interestingly, PTC did not rule out potential future combinations with other novel agents in expanded CF populations (i.e., Vertex's Kalydeco, etc). SMA program – Recall, last week, it was announced a lead development candidate was selected, though timelines for entering the clinic are unknown at this point. A \$10M milestone payment from Roche will be recorded in 3Q13 related to the program.
- **Adjusting estimates:** Based on 2Q results, we are making adjustments to our model. Our 2013-2015 GAAP loss per share estimates change to -\$3.41, -\$2.68, and -\$3.09, respectively, from -\$3.54, -\$2.75, and -\$3.17.
- **Reiterate Overweight rating.** Roche covered by J.P. Morgan analyst Richard Vossler.

Overweight

PTCT, PTCT US

Price: \$15.00

Price Target: \$20.00

Biotechnology

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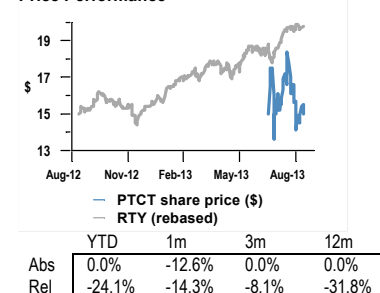
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Price Performance



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)	-	-	(0.77)	(0.59)A	-	-	-	-
Q3 (Sep)	-	-	(0.66)	(0.64)	-	-	-	-
Q4 (Dec)	-	-	(0.77)	(0.68)	-	-	-	-
FY	5.39	7.75	(3.54)	(3.41)	(2.75)	(2.68)	(3.17)	(3.09)

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

Company Data

Price (\$)	15.00
Date Of Price	13 Aug 13
52-week Range (\$)	18.50-13.04
Market Cap (\$ mn)	426.48
Fiscal Year End	Dec
Shares O/S (mn)	28
Price Target (\$)	20.00
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 2Q results, we are making adjustments to our model. Our 2013-2015 GAAP loss per share estimates change to -\$3.41, -\$2.68, and -\$3.09, respectively, from -\$3.54, -\$2.75, and -\$3.17.

Table 1: Changes to Our Model

	2013		2014		2015	
	Old	New	Old	New	Old	New
Total Revenues	25.1	26.0	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	51.2	55.0	56.3	60.5	61.9	66.5
Sales, general and administrative	28.5	26.1	29.9	27.4	35.9	32.8
Operating Income	-54.5	-55.0	-76.2	-77.8	-90.8	-92.3
Net Income	-56.1	-56.4	-74.7	-76.3	-89.3	-90.8
Diluted GAAP EPS	-3.54	-3.41	-2.75	-2.68	-3.17	-3.09

Source: 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 Duchenne 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 December 2014 price target of \$20 for PTCT is based on our sum-of-the-parts analysis including ataluren in nmDMD only. We project ataluren nmDMD sales to 2024, consistent with IP protection, assume no terminal value and a 10% discount rate. We further assume a 70% 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 \$19/share for ataluren. This, taken in combination with net cash of \$1/share, supports our December 2014 PT of \$20.

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	3Q13E	4Q13E
Revenues	34	26	10	7	Revenues	7A	7A	6	6
Cost of products sold	0	0	0	0	Cost of products sold	0A	0A	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(15)	(26)	(27)	(33)	SG&A	(4)A	(7)A	(7)	(8)
R&D	(46)	(55)	(60)	(67)	R&D	(11)A	(15)A	(15)	(15)
Operating income	(27)	(55)	(78)	(92)	Operating income	(9)A	(14)A	(16)	(17)
EBITDA	(27)	(55)	(78)	(92)	EBITDA	(9)A	(14)A	(16)	(17)
Net interest (income) / expense	(1)	(2)	1	1	Net interest (income) / expense	(1)A	(0)A	(1)	(1)
Other income / (expense)	2	0	1	1	Other income / (expense)	0A	(0)A	0	0
Income taxes	0	0	0	0	Income taxes	0A	0A	0	0
Net income - GAAP	(26)	(56)	(76)	(91)	Net income - GAAP	(9)A	(15)A	(16)	(17)
Net income - recurring	133	(71)	(76)	(91)	Net income - recurring	(24)A	(15)A	(16)	(17)
Diluted shares outstanding	17	21	28	29	Diluted shares outstanding	16A	25A	25	25
EPS - excluding non-recurring	(1.52)	(2.70)	(2.68)	(3.09)	EPS - excluding non-recurring	(0.57)A	(0.59)A	(0.64)	(0.68)
EPS - recurring	7.75	(3.41)	(2.68)	(3.09)	EPS - recurring	(1.51)A	(0.59)A	(0.64)	(0.68)
Balance Sheet and Cash Flow Data	FY12A	FY13E	FY14E	FY15E	Ratio Analysis	FY12A	FY13E	FY14E	FY15E
Cash and cash equivalents	3	133	137	46	Sales growth	(67.8%)	(23.4%)	(61.5%)	(30.0%)
Accounts receivable	-	-	-	-	EBIT growth	(187.7%)	105.3%	41.4%	18.7%
Inventories	-	-	-	-	EPS growth - recurring	43.7%	(144.0%)	(21.3%)	15.0%
Other current assets	2	2	2	2	Gross margin	-	-	-	-
Current assets	5	135	139	48	EBIT margin	(79.0%)	(211.7%)	(778.2%)	(1319.2%)
PP&E	8	9	9	9	EBITDA margin	(79.0%)	(211.7%)	(778.2%)	(1319.2%)
Total assets	13	143	148	58	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	5	5	5	5	Net margin	392.8%	(274.1%)	(763.2%)	(1297.8%)
Total liabilities	32	32	32	32	Net Debt / EBITDA	(8.0%)	232.3%	170.4%	44.9%
Shareholders' equity	(19)	111	117	26	Net Debt / Capital (book)	(12.9%)	781.4%	825.6%	262.9%
Net income (including charges)	(26)	(71)	(76)	(91)	Return on assets (ROA)	466.1%	(91.1%)	(52.3%)	(88.2%)
D&A	3	3	3	3	Return on equity (ROE)	(619.6%)	(153.8%)	(67.0%)	(127.8%)
Change in working capital	(25)	0	0	0	Enterprise value / sales	-	-	-	-
Other	1	0	0	0	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	(48)	(69)	(74)	(88)	Free cash flow yield	(18.2%)	(22.3%)	(17.7%)	(20.4%)
Capex	(0)	(3)	(3)	(3)					
Free cash flow	(47)	(70)	(76)	(90)					
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

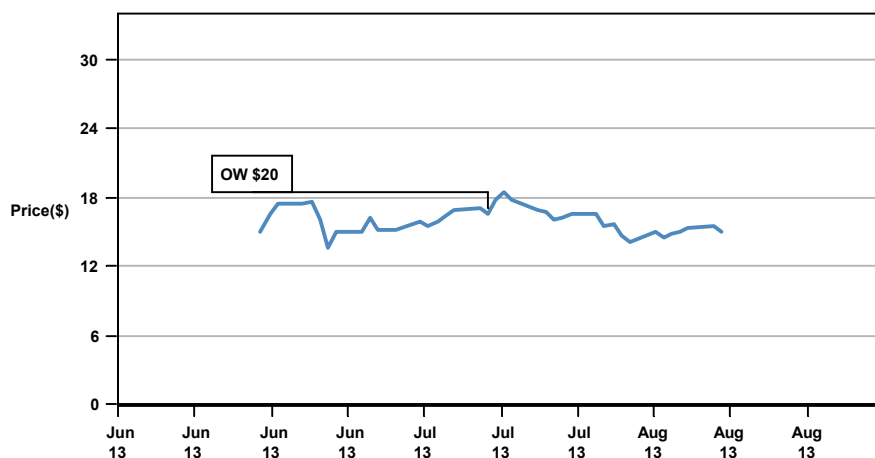
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PTC Therapeutics (PTCT, PTCT US) Price Chart



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

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