

PTC Therapeutics

Our Thoughts Post the 1Q14 Call

There wasn't a lot new on PTC's 1Q call today; the pipeline continues to progress with ataluren nearing completion of enrollment in the phase 3 trial in DMD (guidance: mid 2014) and the CF phase 3 trial soon to be initiated (guidance: 2Q14). Given the lessons learned from the phase 2 experience, we continue to believe that ataluren has a high probability of success in both nmDMD and nmCF, which is captured in the phase 3 design. Of note, given its mechanism of action, ataluren also has potential in multiple indications beyond DMD and CF with the start of a phase 2 proof-of concept study in an undisclosed indication occurring in 2H14. While a final decision on conditional approval of ataluren in nmDMD in Europe is still expected in 2Q14, we continue to view this as a low probability event (given the negative CHMP opinion) which notably isn't reflected in our operating model. Overall, we are encouraged by the continued pipeline progress and given the opportunity for ataluren (\$700M nmDMD alone), we reiterate our Overweight rating.

- **Ataluren in nmDMD:** The ongoing phase 3 ACT DMD trial of ataluren in nmDMD remains on track to complete enrollment in mid-2014 with data expected a year later in mid 2015. Additionally, a final decision from the EMA on conditional approval of ataluren in nmDMD is still expected in 2Q14. Recall, the CHMP issued a negative opinion in January and we continue to believe approval will likely require data from the phase 3 ACT DMD trial.
- **Ataluren in nmCF:** PTC is in the process of initiating a phase 3 ACT CF confirmatory trial in 2Q14, with FEV1 as the primary endpoint and pulmonary exacerbations as a key secondary endpoint. Importantly, patients on tobramycin will be excluded from the study, as previously noted. Recall, in the phase 2 study, tobramycin, a known ribosome binder, confounded the results. Given exclusion of these patients, we anticipate a positive outcome in the phase 3 ACT CF trial though this represents upside to our model.
- **Adjusting estimates:** Following the quarter and recent trends we are updating our model. We have fine tuned operating expenses and increased shares outstanding. The net impact is our 2014-2016 GAAP EPS estimates change to (\$2.83), (\$3.02) and (\$3.17), from (\$2.79), (\$3.15) and (\$3.30), respectively.

Reiterate Overweight rating.

PTC Therapeutics (PTCT;PTCT US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)	2015E (Prev)	2015E (Curr)	2016E (Prev)	2016E (Curr)
EPS Reported (\$)							
Q1 (Mar)	(1.51)	(0.44)A	(0.58)A	-	-	-	-
Q2 (Jun)	(0.59)	(0.72)	(0.69)	-	-	-	-
Q3 (Sep)	(0.19)	(0.78)	(0.75)	-	-	-	-
Q4 (Dec)	(0.75)	(0.82)	(0.79)	-	-	-	-
FY	(2.75)	(2.79)	(2.83)	(3.15)	(3.02)	(3.30)	(3.17)

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

Overweight

PTCT, PTCT US

Price: \$18.67

Price Target: \$40.00

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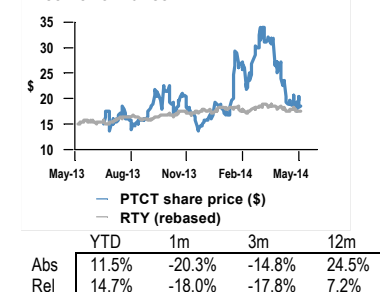
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Price Performance



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Changes to Our Model

Following the quarter and recent trends we are updating our model. We have fine tuned operating expenses and increase shares outstanding. The net impact is our 2014-2016 GAAP PES estimates change to (\$2.83), (\$3.02) and (\$3.17), from (\$2.79), (\$3.15) and (\$3.30), respectively

Table 1: Changes to Our Model

	2014		2015		2016	
	Old	New	Old	New	Old	New
Total Revenues	23.5	21.2	22.0	22.0	34.8	34.8
Cost of sales	0.0	0.0	0.0	0.0	2.5	2.5
Research and development	69.5	69.6	76.5	76.6	84.1	84.3
Sales, general and administrative	34.1	33.8	40.9	40.6	57.3	56.8
Operating Income	-80.1	-82.2	-95.4	-95.2	-109.1	-108.8
Net Income	-78.6	-81.0	-93.9	-93.7	-106.6	-106.3
Diluted GAAP EPS	-2.79	-2.83	-3.15	-3.02	-3.30	-3.17

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	1Q14A	2Q14E	3Q14E	4Q14E
Revenues	35	21	22	35	Revenues	9A	4	4	4
Cost of products sold	0	0	0	(2)	Cost of products sold	0A	0	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(25)	(34)	(41)	(57)	SG&A	(8)A	(8)	(9)	(9)
R&D	(55)	(70)	(77)	(84)	R&D	(16)A	(17)	(18)	(19)
Operating income	(45)	(82)	(95)	(109)	Operating income	(14)A	(21)	(23)	(24)
EBITDA	(45)	(82)	(95)	(109)	EBITDA	(14)A	(21)	(23)	(24)
Net interest (income) / expense	(1)	1	1	2	Net interest (income) / expense	0A	0	0	0
Other income / (expense)	0	0	1	1	Other income / (expense)	(0)A	0	0	0
Income taxes	0	0	0	0	Income taxes	0A	0	0	0
Net income - GAAP	(46)	(81)	(94)	(106)	Net income - GAAP	(14)A	(21)	(22)	(24)
Net income - recurring	(61)	(81)	(94)	(106)	Net income - recurring	(14)A	(21)	(22)	(24)
Diluted shares outstanding	22	29	31	33	Diluted shares outstanding	24A	30	30	30
EPS - excluding non-recurring	(2.08)	(2.83)	(3.02)	(3.17)	EPS - excluding non-recurring	(0.58)A	(0.69)	(0.75)	(0.79)
EPS - recurring	(2.75)	(2.83)	(3.02)	(3.17)	EPS - recurring	(0.58)A	(0.69)	(0.75)	(0.79)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	143	172	78	76	Sales growth	2.2%	(38.8%)	3.7%	58.1%
Accounts receivable	-	-	-	-	EBIT growth	69.3%	81.2%	15.7%	14.3%
Inventories	-	-	-	-	EPS growth - recurring	(135.5%)	2.9%	6.8%	5.0%
Other current assets	2	2	2	2	Gross margin	-	-	-	-
Current assets	145	174	80	78	EBIT margin	(130.8%)	(387.6%)	(432.6%)	(312.8%)
PP&E	9	9	9	9	EBITDA margin	(130.8%)	(387.6%)	(432.6%)	(312.8%)
Total assets	154	183	89	88	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	5	5	5	5	Net margin	(175.1%)	(381.8%)	(425.8%)	(305.6%)
Total liabilities	32	32	32	32	Net Debt / EBITDA	304.7%	203.4%	77.0%	65.6%
Shareholders' equity	122	151	58	56	Net Debt / Capital (book)	845.6%	1041.6%	465.0%	461.4%
Net income (including charges)	(61)	(81)	(94)	(106)	Return on assets (ROA)	(72.8%)	(48.1%)	(68.8%)	(120.0%)
D&A	3	3	3	3	Return on equity (ROE)	(117.8%)	(59.3%)	(89.8%)	(187.4%)
Change in working capital	0	0	0	0	Enterprise value / sales	-	-	-	-
Other	0	0	0	0	Enterprise value / EBITDA	-	-	-	-
Cash flow from operations	(58)	(78)	(91)	(104)	Free cash flow yield	(14.7%)	(15.0%)	(16.1%)	(16.7%)
Capex	(3)	(3)	(3)	(3)					
Free cash flow	(61)	(80)	(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

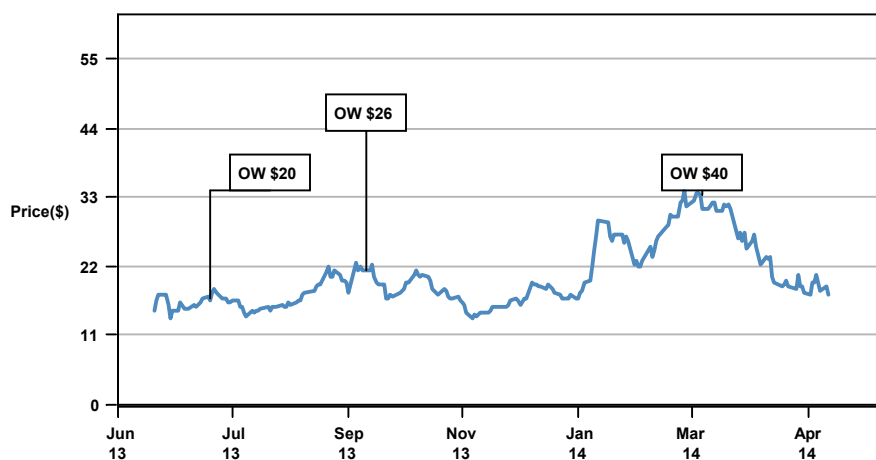
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PTC Therapeutics (PTCT, PTCT US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
15-Jul-13	OW	16.94	20.00
27-Sep-13	OW	21.37	26.00
06-Mar-14	OW	33.38	40.00

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

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