

PTC Therapeutics, Inc (PTCT)

SMALL & MID CAP RESEARCH

All Programs On Track - Reiterate Outperform

PTCT remains on track and well-funded for two Phase III readouts for Ataluren in DMD in 2015 and CF in 2016. We believe there is currently nothing in the stock for a potential early EU approval of Ataluren, making the Q2 EMA decision a relatively cheap/free option. Disclosure of a third indication for Ataluren in H2:14 and early Phase I data for the SMA program in late 2014/2015, could provide some nearer term clinical validation of its broader portfolio.

- **In-line quarter:** PTCT reported revenues and earnings relatively in-line with our expectations. Q1 revenues were \$9.2M vs CS est of \$9.5M, and EPS was (\$0.58) vs. CS est of (\$0.54). We made minor adjustments to our forward EPS estimates.
- **Well-funded:** PTCT ended Q1 with \$246.6M, sufficient to fund operations through 2016 and Phase III results in DMD and CF.
- **Key catalysts:** (1) Re-examination of European filing in Q2:14; (2) complete enrollment in DMD Phase III in mid-2014, (3) SMA Phase I readout in 2014/15, (4) DMD Phase III read out in mid-2015, and (5) CF Phase III read out in 2016.
- **\$35 target – Conservative if data is positive:** Our valuation includes ataluren (\$23), SMA program (\$6), and one-year forward net cash (\$6). We use a 65% probability of success for ataluren, suggesting significant upside on positive data, and comps in the orphan disease space – VRTX, BMRN, ALXN, etc. – support multi-billion dollar valuations for rare disease franchises.

Rating	OUTPERFORM* [V]
Price (06 May 14, US\$)	17.44
Target price (US\$)	35.00 ¹
52-week price range	33.97 - 13.59
Market cap. (US\$ m)	524.54
Enterprise value (US\$ m)	335.47

*Stock ratings are relative to the coverage universe in each analyst's or each team's respective sector.

¹Target price is for 12 months.

[V] = Stock considered volatile (see Disclosure Appendix).

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Financial and valuation metrics

Year	12/13A	12/14E	12/15E	12/16E
EPS (CS adj.) (US\$)	-5.18	-2.93	-2.90	-2.49
Prev. EPS (US\$)	—	-2.92	-3.02	-2.59
P/E (x)	-3.4	-5.9	-6.0	-7.0
P/E rel. (%)	-19.6	-37.4	-42.2	-54.6
Revenue (US\$ m)	34.7	18.2	22.0	47.7
EBITDA (US\$ m)	-43.0	-79.9	-85.4	-87.0
OCFPS (US\$)	-3.66	-2.35	-2.20	-1.90
P/OCF (x)	-4.6	-7.4	-7.9	-9.2
EV/EBITDA (current)	-6.5	-3.5	-3.3	-3.2
Net debt (US\$ m)	-142	-189	-118	-231
ROIC (%)	760.27	3,907.16	2,965.90	2,369.89
Number of shares (m)	30.08	IC (current, US\$ m)		-5.97
BV/share (Next Qtr., US\$)	-12.5	EV/IC (x)		-153.2
Net debt (Next Qtr., US\$ m)	-228.8	Dividend (current, US\$)		—
Net debt/tot cap (Next Qtr., %)	-100.9	Dividend yield (%)		—

Source: Company data, Credit Suisse estimates

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Exhibit 1: Q1:14 Variance Table

Income Statement	Actual	CS Estimate	Difference
Collaboration revenue	9.1	9.5	(0.4)
Grant revenue	0.1	0.0	0.1
Total Revenues	9.2	9.5	(0.3)
COGS	0.0	0.0	0.0
Research and Development Expenses	15.9	15.3	0.6
Sales, General and Administrative Expenses	7.5	7.5	0.0
Total Costs and Expenses	23.4	22.8	0.6
Operating Income (Loss)	(14.2)	(13.3)	(0.9)
Interest Expense, net	0.2	0.0	0.2
Other income, net	(0.1)	(1.0)	0.9
Income (Loss) before Tax	(14.1)	(14.3)	0.2
Provision for Income Tax (benefit)	0.0	0.0	0.0
Net income (loss)	(14.1)	(14.3)	0.2
Net income attributable to common shareholders	(14.1)	(14.3)	0.2
Basic Loss per Common Share	(0.58)	(0.54)	(0.04)
Diluted Loss per Common Share	0.0	0.0	0.0
Shares Outstanding, Basic	24.5	26.5	(2.0)
Shares Outstanding, Diluted*	26.7	28.7	(4.2)

Source: Company data, Credit Suisse estimates

Exhibit 2: PTCT News Flow

Product	Indication	Catalyst	Expected Date
Ataluren	Cystic Fibrosis	CHMP decision on re-examination	Q2:14
Ataluren	Cystic Fibrosis	Initiate Phase III trial	Q2:14
Ataluren	Duchenne Muscular Dystrophy	Complete Phase III enrollment	Mid-2014
Ataluren	Duchenne Muscular Dystrophy	Data from EU open-label extension study	H2:14
Ataluren	3rd indication	Initiate Phase I testing	2014
SMA program	Spinal muscular atrophy	Complete Phase I	YE:2014
Ataluren	Duchenne Muscular Dystrophy	Phase III data	Mid-2015
Ataluren	Cystic Fibrosis	Complete Phase III enrollment	Mid-2015
Ataluren	Duchenne Muscular Dystrophy	FDA and EMA filing for full approval	Late-2015
Ataluren	Duchenne Muscular Dystrophy	Potential FDA and EMA approval	Mid-2016
Ataluren	Cystic Fibrosis	Phase III data	Mid-2016
Ataluren	Cystic Fibrosis	FDA and MAA filing for full approval	YE:16/early 2017
Ataluren	Cystic Fibrosis	Potential FDA and EMA approval	Mid-2017

Source: Company data, Credit Suisse estimates

Exhibit 3: PTCT pipeline

Product/Indication	Phase	Target	Partner
Ataluren - Duchenne Muscular Dystrophy	Phase III; MAA submitted	Nonsense DMD mutations	Proprietary
Ataluren - Cystic Fibrosis	Phase III ready	Class 1 CFTR Mutations	Proprietary
Spinal muscular atrophy	Phase I	SMN2	Roche
PTC596 - Oncology	Preclinical	BMI1	Proprietary
Antibacterial	Preclinical	MDR Gram (-) bacteria	Proprietary

Source: Company data, Credit Suisse estimates

Exhibit 4: 2014 Guidance

	Guidance	CS est.
Operating expenses (excluding SOE)	\$85-95M	\$86
Cash at year-end 2014	\$175-185M	\$189

Source: Company data, Credit Suisse estimates

Exhibit 5: PTCT model

(\$ in MM; except per share)	2011A	2012A	2013A	Q1:14A	Q2:14E	Q3:14E	Q4:14E	2014E	2015E	2016E	2017E	2018E
US Sales										16.9	110.7	189.2
EU Sales										14.8	56.1	124.0
ROW Royalties											2.9	7.6
Ataluren revenue (total)										31.7	169.6	320.8
Collaboration revenue	99.0	28.8	31.3	9.1	2.0	2.0	2.0	15.1	18.0	16.0	16.0	12.0
Grant revenue	6.5	5.2	3.4	0.1	1.0	1.0	1.0	3.1	4.0			
Total Revenues	105.4	33.9	34.7	9.2	3.0	3.0	3.0	18.2	22.0	47.7	185.6	332.8
COGS										2.5	13.6	25.7
Research and Development Expenses	58.7	46.1	54.9	15.9	16.3	18.3	19.3	69.8	73.5	66.0	70.0	77.0
Sales, General and Administrative Expenses	16.2	14.6	25.2	7.5	7.6	7.7	7.8	30.8	34.7	67.0	101.0	119.2
Total Costs and Expenses	74.8	60.8	80.1	23.4	23.9	26.0	27.1	100.5	108.2	135.5	184.6	221.9
Operating Income (Loss)	30.6	(26.8)	(45.4)	(14.2)	(20.9)	(23.0)	(24.1)	(82.3)	(86.2)	(87.8)	1.1	110.9
Interest Expense, net	(2.4)	(1.2)	(6.1)	0.2				0.2				
Other income, net	0.5	1.8	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.4)	(0.4)	(0.4)	(1.0)	(1.0)
Income (Loss) before Tax	28.6	(26.2)	(51.6)	(14.1)	(21.0)	(23.1)	(24.2)	(82.5)	(86.6)	(88.2)	0.1	109.9
Provision for Income Tax (benefit)	2.3											
Net income (loss)	30.9	(26.2)	(51.6)	(14.1)	(21.0)	(23.1)	(24.2)	(82.5)	(86.6)	(88.2)	0.1	109.9
Net income attributable to common shareholders	0.0	0.7	(66.4)	(14.1)	(21.0)	(23.1)	(24.2)	(82.5)	(86.6)	(88.2)	0.1	109.9
EPS - diluted	4.55	42.50	(5.18)	(0.6)	(0.7)	(0.8)	(0.8)	(2.93)	(2.90)	(2.49)	0.00	2.75
Shares Outstanding - basic	0.001	0.003	12.83	24.49	29.20	29.35	29.50	28.14	29.87	35.51	36.22	36.95
Shares Outstanding - diluted	0.006	0.017	12.83	26.66	31.41	31.60	31.79	30.37	32.28	38.12	39.05	40.01

Source: Company data, Credit Suisse estimates

Companies Mentioned (Price as of 06-May-2014)

PTC Therapeutics, Inc (PTCT.OQ, \$17.44, OUTPERFORM[V], TP \$35.0)

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3-Year Price and Rating History for PTC Therapeutics, Inc (PTCT.OQ)

PTCT.OQ	Closing Price	Target Price	
Date	(US\$)	(US\$)	Rating
15-Jul-13	17.17	24.00	O *
31-Jan-14	26.07		R
18-Feb-14	28.43	35.00	O

* Asterisk signifies initiation or assumption of coverage.



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Price Target: (12 months) for PTC Therapeutics, Inc (PTCT.OQ)

Method: Our \$35 target price for PTCT is calculated by DCF (discounted cash flow), using probability-weighted sales estimates for ataluren in Duchenne muscular dystrophy (65% probability) and in cystic fibrosis (65% probability) modeled through 2030, and the SMA program (20% probability). We use a 38% tax rate and a 12% discount rate, and arrive at a \$35 valuation based on a projected share count.

Risk: Risks to our \$35 target price for PTCT are (1) unexpected negative result in the Duchenne muscular dystrophy (DMD) or cystic fibrosis (CF) Phase III studies, (2) headline risk should the EMA (European Medicines Agency) reject conditional approval of ataluren in DMD, (3) limited newsflow in 2014, (4) potential emergence a competitive molecule in the DMD or CF space, and (5) potential need for additional capital (we model an equity raise in 2016).

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