PTC Therapeutics, Inc (PTCT)

Rating	**OUTPERFORM* [V]**
Price (06 Mar 14, US$)	33.38
Target price (US$)	35.00[1]
52-week price range	33.97 - 13.59
Market cap. (US$ m)	1,003.96
Enterprise value (US$ m)	814.93

**Stock ratings are relative to the coverage universe in each analyst's or each team's respective sector.*
[1]Target price is for 12 months.
[V] = Stock considered volatile (see Disclosure Appendix).

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SMALL & MID CAP RESEARCH

Remain Bullish on Ataluren and PTCT Platform

PTCT remains one of our top small cap ideas based on the large open-ended opportunity across multiple orphan genetic diseases, its retained products rights, ongoing and planned Phase III trials, and significant upside potential if Phase III data is positive in 2015. Current funding is sufficient to reach key milestones in four clinical programs – DMD, CF, SMA, and an undisclosed new indication for ataluren. Increased operating expenses based on 2014 guidance lead us to lower our EPS for 2014 and 2015.

- **Well-funded.** Following its recent stock issuance PTCT had proforma cash over $250M, 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 ($22), SMA program ($6), and net cash ($7). 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.
- **Increased expenses.** PTCT guided to cash operating expenses of $85-95M. We increased R&D expenses in 2014 and 2015, and our EPS estimates decrease to ($2.92) and ($3.02), respectively. Our year-end 2014 cash estimate of $189M is slightly ahead of guidance.

Financial and valuation metrics

Year	12/13A	12/14E	12/15E	12/16E
EPS (CS adj.) (US$)	-5.18	-2.92	-3.02	-2.59
Prev. EPS (US$)	—	-2.09	-2.09	-2.42
P/E (x)	-6.4	-11.4	-11.1	-12.9
P/E rel. (%)	-38.3	-73.8	-79.0	-102.8
Revenue (US$ m)	34.7	18.5	22.0	47.7
EBITDA (US$ m)	-43.0	-77.2	-85.4	-87.0
OCFPS (US$)	-3.66	-2.30	-2.34	-2.02
P/OCF (x)	-4.6	-14.5	-14.3	-16.5
EV/EBITDA (current)	-20.0	-11.2	-10.1	-9.9
Net debt (US$ m)	-142	-189	-114	-223
ROIC (%)	760.27	1,791.76	1,645.99	1,454.98

Number of shares (m)	30.08	IC (current, US$ m)	-5.97
BV/share (Next Qtr., US$)	-12.9	EV/IC (x)	-161.5
Net debt (Next Qtr., US$ m)	-248.0	Dividend (current, US$)	—
Net debt/tot cap (Next Qtr., %)	-101.9	Dividend yield (%)	—

Source: Company data, Credit Suisse estimates

Exhibit 1: Q4:13 variance table

Income Statement	Actual	CS Estimate	Difference
Collaboration revenue	3.9	5.0	(1.1)
Grant revenue	0.5	1.0	(0.5)
Total Revenues	**4.4**	**6.0**	**(1.6)**
COGS	0.0	0.0	0.0
Research and Development Expenses	15.0	12.0	3.0
Sales, General and Administrative Expenses	7.5	6.5	1.0
Total Costs and Expenses	22.5	18.5	4.0
Operating Income (Loss)	**(18.1)**	**(12.5)**	**(5.6)**
Interest Expense, net	0.2	(0.0)	0.2
Other income, net	0.0	(1.0)	1.0
Income (Loss) before Tax	(17.9)	(13.5)	(4.3)
Provision for Income Tax (benefit)	0.0	0.0	0.0
Net income (loss)	**(17.9)**	**(13.5)**	**(4.3)**
Net income attributable to common shareholders	(17.9)	(13.5)	(4.3)
Basic Loss per Common Share	**(0.75)**	**(0.54)**	**(0.21)**
Diluted Loss per Common Share	0.0	0.0	0.0
Shares Outstanding, Basic	23.8	24.9	(1.1)
Shares Outstanding, Diluted*	23.8	27.0	(3.2)

Source: Company data, Credit Suisse estimates

Exhibit 2: PTCT news flow

Product/ Event	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	$84
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	Q1:14E	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	9.5	2.0	2.0	2.0	15.5	18.0	16.0	16.0	12.0
Grant revenue	6.5	5.2	3.4		1.0	1.0	1.0	3.0	4.0			
Total Revenues	105.4	33.9	34.7	9.5	3.0	3.0	3.0	18.5	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	15.3	15.7	17.7	18.7	67.4	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	30.6	34.7	67.0	101.0	119.2
Total Costs and Expenses	74.8	60.8	80.1	22.8	23.3	25.4	26.5	98.0	108.2	135.5	184.6	221.9
Operating Income (Loss)	30.6	(26.8)	(45.4)	(13.3)	(20.3)	(22.4)	(23.5)	(79.5)	(86.2)	(87.8)	1.1	110.9
Interest Expense, net	(2.4)	(1.2)	(6.1)									
Other income, net	0.5	1.8	(0.1)	(1.0)	(1.0)	(1.0)	(1.0)	(4.0)	(4.0)	(4.0)	(4.0)	(4.0)
Income (Loss) before Tax	28.6	(26.2)	(51.6)	(14.3)	(21.3)	(23.4)	(24.5)	(83.5)	(90.2)	(91.8)	(2.9)	106.9
Provision for Income Tax (benefit)	2.3											
Net income (loss)	30.9	(26.2)	(51.6)	(14.3)	(21.3)	(23.4)	(24.5)	(83.5)	(90.2)	(91.8)	(2.9)	106.9
Net income attributable to common shareholders	0.0	0.7	(66.4)	(14.3)	(21.3)	(23.4)	(24.5)	(83.5)	(90.2)	(91.8)	(2.9)	106.9
EPS - diluted	4.55	42.50	(5.18)	(0.54)	(0.73)	(0.80)	(0.83)	(2.92)	(3.02)	(2.59)	(0.08)	2.67
Shares Outstanding - basic	0.001	0.003	12.83	26.50	29.20	29.35	29.50	28.64	29.87	35.51	36.22	36.95
Shares Outstanding - diluted	0.006	0.017	12.83	28.67	31.41	31.60	31.79	30.87	32.28	38.12	39.05	40.01

Source: Company data, Credit Suisse estimates

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Companies Mentioned *(Price as of 06-Mar-2014)*

PTC Therapeutics, Inc (PTCT.OQ, $33.38, OUTPERFORM[V], TP $35.0)

Disclosure Appendix

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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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Underperform/Sell*	15%	(43% banking clients)
Restricted	2%	

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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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