PTC Therapeutics, Inc (PTCT)

Rating	**OUTPERFORM* [V]**
Price (14 Feb 14, US$)	26.62
Target price (US$)	(from 24.00) 35.00[1]
52-week price range	29.32 - 13.59
Market cap. (US$ m)	663.29
Enterprise value (US$ m)	516.01

**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

New Financing Secures Funding for Multiple Key Data Events

PTCT raised gross proceeds of $110M ($126.5M if the overallotment is exercised), which should now fund the company through (1) Phase III readouts for both DMD in 2015 and CF in 2016, (2) proof of concept trials for Ataluren in one or two additional genetic diseases, (3) advancement of the SMA program with Roche, and (4) advancement of the BM1 program in cancer which should enter clinical testing in H1:14. Due to the recent progress in the SMA program, we now include it in our valuation, which raises our TP to $35 from $24.

- **Increasing TP to $35 from $24:** We have added the SMA program to our valuation ($6), which also includes Ataluren ($22), and net cash ($7). We increased our Ataluren valuation by increasing POS to 65% from 60%. We include the newly issued shares from the recent equity raise, which we had previously modeled in 2015. The result is an increase to our 2014 EPS estimate and a decrease in 2015.
- **SMA could be a significant value driver.** An ongoing Phase I trial could provide initial proof of concept biomarker data (in healthy volunteers) in the next 12 months. If successful, we believe SMA could be a multi-billion dollar global opportunity. PTCT is partnered with Roche.
- **Catalysts:** (1) Re-examination 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.

Financial and valuation metrics

Year	12/12A	12/13E	12/14E	12/15E
EPS (CS adj.) (US$)	42.50	-3.68	-2.09	-2.09
Prev. EPS (US$)	—	—	-2.47	-1.92
P/E (x)	0.6	-7.2	-12.7	-12.7
P/E rel. (%)	3.6	-43.6	-84.2	-93.5
Revenue (US$ m)	33.9	36.3	18.5	22.0
EBITDA (US$ m)	-24.1	-37.4	-55.9	-60.0
OCFPS (US$)	NM	-3.18	-1.81	-1.58
P/OCF (x)	—	-8.4	-14.7	-16.9
EV/EBITDA (current)	-21.0	-13.5	-9.0	-8.4
Net debt (US$ m)	2	-147	-207	-154
ROIC (%)	160.91	509.10	3,724.92	2,551.66

Number of shares (m)	24.92	IC (current, US$ m)	-16.66
BV/share (Next Qtr., US$)	-13.0	EV/IC (x)	-66.0
Net debt (Next Qtr., US$ m)	-147.3	Dividend (current, US$)	—
Net debt/tot cap (Next Qtr., %)	-105.6	Dividend yield (%)	—

Source: Company data, Credit Suisse estimates

Updates from PTCT

CHMP appeal process for Ataluren in DMD: PTCT disclosed that the negative opinion consisted of a split vote and that one of the major issues raised was the potential impact of an approval on the enrollment of the Phase III trial. PTCT has filed for a re-examination. This involves the addition of two new rapporteurs to the review process, which should be completed in late Q2:14. At that time, the Phase III trial should be nearly completely enrolled, which may give regulators more comfort in an early approval.

EU regulatory update for Ataluren in CF: PTCT had previously planned to file for an early conditional approval of Ataluren in CF based on the previously completed Phase III study. PTCT now plans to wait until complete resolution of the DMD filing before considering another application. The rationale is to avoid doing anything that could disrupt the current process. Also, given the feedback in DMD regarding completing a confirmatory Phase III trial, it seems to us that a filing in CF would likely be substantially later, given that the confirmatory study is scheduled to initiate in Q2:14.

SMA program: In partnership with Roche, there are now three SMA trials ongoing. The first is a standard Phase I trial in 48 healthy volunteers to measure safety, PK, and potentially biomarkers of activity (SMN RNA and protein levels). The other two studies include a biomarker study and a natural history study (neither include drug treatment), which are both designed to develop the tools and information needed to conduct a robust Phase II and III program.

New proof of concept studies for Ataluren: One of the uses of proceeds in the recent financing was to fund expanded development of Ataluren in other non-sense mutation genetic diseases. While PTCT has not committed to which specific diseases they would target, they did highlight several disease where Ataluren has been tested preclinically including Hurler's syndrome, aniridia, and choroidermia.

Now including SMA in our valuation

Our new valuation includes a slightly higher probability of success for Ataluren (65% vs. 60% in both CF and DMD) and now includes a probability adjusted value for the SMA program. Our new price target implies a $1b market cap based on the new share count.

Exhibit 1: PTCT sum-of-the-parts valuation

DCF valuation		Value
Ataluren	667	$22
SMA program	191	$6
Net Cash	203	$7
Total	1,061	$35

Source: Company data, Credit Suisse estimates

Exhibit 2: Orphan Genetic Diseases Companies

Company	Ticker	Price 2/14/14	Lead Product	Stage	Shares	Cash	Debt	Mark. Cap	Ent. Val
Alexion Pharmaceuticals, Inc.	ALXN	$180.82	Soliris	Marketed	197.0	1,514.9	145.2	35,613.6	34,244.0
Vertex Pharmaceuticals	VRTX	$81.39	Kalydeco	Marketed	233.8	1,465.1	0.0	19,029.0	17,563.9
BioMarin Pharmaceutical Inc.	BMRN	$76.25	Naglazyme, etc	Marketed	142.2	379.7	78.3	10,842.4	10,541.0
Isis Pharmaceuticals	ISIS	$52.62	Kynamro	Marketed	116.0	737.7	159.7	6,103.8	5,525.8
Alnylam	ALNY	$78.55	ALN-TTR02	Phase II	63.2	193.8	0.0	4,967.2	4,773.4
Synageva BioPharma Corp.	GEVA	$99.82	Sebelipase alfa	Phase III	30.7	438.4	0.0	3,063.0	2,624.6
Aegerion Pharmaceuticals, Inc.	AEGR	$63.23	Lomitapide	Marketed	29.2	126.2	8.5	1,844.5	1,726.8
Sarepta Therapeutics Inc.	SRPT	$26.35	Eteplirsen	Phase II	37.5	280.9	1.7	989.0	709.8
Raptor Pharmaceutical Corp.	RPTP	$15.10	Procysbi	Marketed	61.1	88.8	50.1	923.3	884.5
PTC Therapeutics	PTCT	$26.46	Ataluren	Phase III	30.1	259.0	0.1	795.6	536.7
Corcept Therapeutics Incorpora	CORT	$3.08	Korlym	Marketed	99.8	63.2	34.6	307.4	278.9
Prosensa	RNA	$6.45	Drisapersen	Phase III	35.9	130.4	8.6	231.6	109.8
Amicus Therapeutics, Inc.	FOLD	$2.68	Migalastat HCl	Phase III	57.1	75.5	25.4	153.1	103.0
Average								***6,528.0***	***6,124.8***
Median								***1,844.5***	***1,726.8***

Source: Company data, Credit Suisse estimates

We add $6/share for the SMA program.

We have made the following assumptions in our SMA valuation:

- 8,000 patients in the US and 10,000 patients in the EU, 2,500 in Japan, and 5,000 in ROW.
- Blended price point of $250,000
- We apply a 6X sales multiple on future sales
- We discount the future sales by 12% over 6 years
- 20% probability of success in the SMA setting
- High single-digit to mid-teen royalty from Roche
- Assume that at least one other SMA product is approved
- Risk adjusted and discounted value of future milestone payments under the Roche collaboration

Future upside to our valuation could be triggered if the Phase I study in healthy volunteers demonstrates convincing evidence that the splicing of the SMN2 gene is enhanced in peripheral blood cells. As there is a dose dependent enhancement of SMN2 splicing in the blood and the brain in animals, we believe enhanced splicing in the blood of healthy volunteers would suggest that the drug is impacting splicing in the CNS as well, in our opinion.

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: PTCT news flow

Product/ Event	Indication	Catalyst	Expected Date	Price Sensitivity
Ataluren	Cystic Fibrosis	CHMP decision for appeal of prior decision	Q2:14	Medium
Ataluren	Cystic Fibrosis	Dose first patient in confirmatory CF Phase III study	Q2:14	Low
Ataluren	Duchenne Muscular Dystrophy	Complete enrollment in confirmatory DMD Phase III study	Mid-2014	Low
Ataluren	Duchenne Muscular Dystrophy	Potential data from EU open-label extension study	H2:14	High
BM1	Cancer	Initiate Phase I testing	H2:14	Low
SMA program	Spinal muscular atrophy	Complete Phase I	YE:2014	Medium
Ataluren	Duchenne Muscular Dystrophy	Potential data from confirmatory DMD Phase III study	Mid-2015	High
Ataluren	Cystic Fibrosis	Complete enrollment in confirmatory CF Phase III study	Mid-2015	Low
Ataluren	Duchenne Muscular Dystrophy	FDA and EMA filing for full approval	Late-2015	Low
Ataluren	Duchenne Muscular Dystrophy	Potential FDA and EMA approval	Mid-2016	High
Ataluren	Cystic Fibrosis	Potential data from confirmatory CF Phase III study	Mid-2016	High
Ataluren	Cystic Fibrosis	FDA and MAA filing for full approval	YE:16/early 2017	Low
Ataluren	Cystic Fibrosis	Potential FDA and EMA approval	Mid-2017	High

Source: Company data, Credit Suisse estimates

Exhibit 5: PTCT Model

Company	Ticker	Price 2/14/14	Lead Product	Stage	Shares	Cash	Debt	Mark. Cap	Ent. Val
Alexion Pharmaceuticals, Inc.	ALXN	$180.55	Soliris	Marketed	197.0	1,514.9	145.2	35,560.4	34,190.8
Vertex Pharmaceuticals	VRTX	$81.61	Kalydeco	Marketed	233.8	1,465.1	0.0	19,079.5	17,614.4
BioMarin Pharmaceutical Inc.	BMRN	$75.81	Naglazyme, etc	Marketed	142.2	379.7	78.3	10,780.3	10,478.9
Isis Pharmaceuticals	ISIS	$52.16	Kynamro	Marketed	116.0	737.7	159.7	6,050.5	5,472.5
Alnylam	ALNY	$78.72	ALN-TTR02	Phase II	63.2	193.8	0.0	4,977.9	4,784.2
Synageva BioPharma Corp.	GEVA	$100.06	Sebelipase alfa	Phase III	30.7	438.4	0.0	3,070.3	2,632.0
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Sarepta Therapeutics Inc.	SRPT	$26.16	Eteplirsen	Phase II	37.5	280.9	1.7	981.9	702.7
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Prosensa	RNA	$6.40	Drisapersen	Phase III	35.9	130.4	8.6	229.8	108.0
Amicus Therapeutics, Inc.	FOLD	$2.67	Migalastat HCl	Phase III	57.1	75.5	25.4	152.5	102.4
Average								***6,519.1***	***6,116.0***
Median								***1,834.9***	***1,717.2***

Source: Company data, Credit Suisse estimates

Companies Mentioned *(Price as of 14-Feb-2014)*

PTC Therapeutics, Inc (PTCT.OQ, $26.62, OUTPERFORM[V], TP $35.0)
Roche (ROG.VX, SFr263.0)

Disclosure Appendix

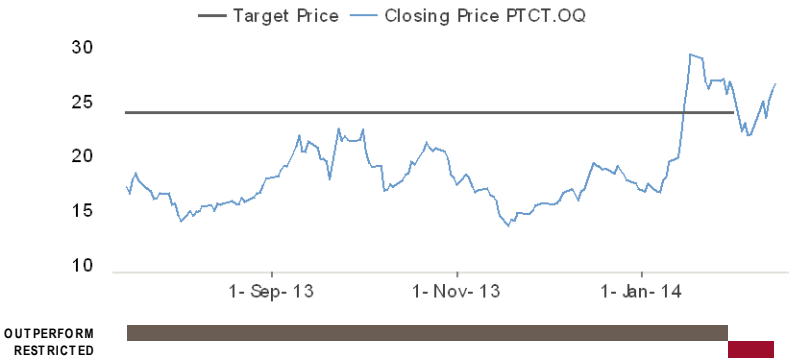
Important Global Disclosures

Jason Kantor, PhD, Ravi Mehrotra PhD and Lee Kalowski each certify, with respect to the companies or securities that the individual analyzes, that (1) the views expressed in this report accurately reflect his or her personal views about all of the subject companies and securities and (2) no part of his or her compensation was, is or will be directly or indirectly related to the specific recommendations or views expressed in this report.

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

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

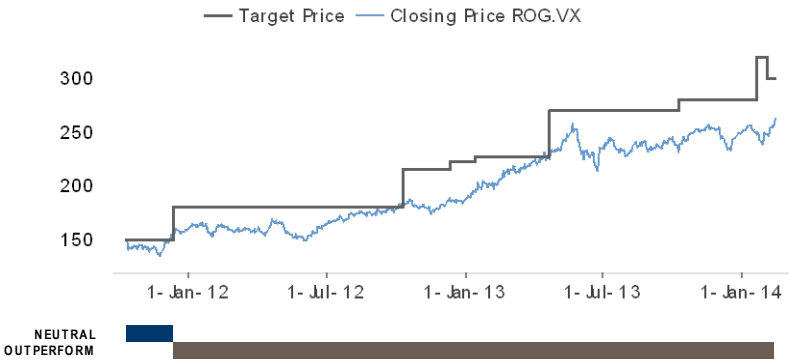
** Asterisk signifies initiation or assumption of coverage.*



3-Year Price and Rating History for Roche (ROG.VX)

ROG.VX Date	Closing Price (SFr)	Target Price (SFr)	Rating
11-Oct-11	148.90	150.00	N
12-Dec-11	153.90	180.00	O
11-Oct-12	181.20	215.00	
12-Dec-12	187.20	223.00	
14-Jan-13	194.60	227.00	
22-Apr-13	225.10	270.00	
10-Oct-13	234.60	280.00	
20-Jan-14	250.00	320.00	
03-Feb-14	248.20	300.00	

** Asterisk signifies initiation or assumption of coverage.*



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Neutral/Hold*	40%	(49% banking clients)
Underperform/Sell*	15%	(44% 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).

Please refer to the firm's disclosure website at https://rave.credit-suisse.com/disclosures for the definitions of abbreviations typically used in the target price method and risk sections.

See the Companies Mentioned section for full company names

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Credit Suisse provided non-investment banking services to the subject company (ROG.VX) within the past 12 months

Credit Suisse has managed or co-managed a public offering of securities for the subject company (PTCT.OQ) within the past 12 months.

Credit Suisse has received investment banking related compensation from the subject company (PTCT.OQ) within the past 12 months

Credit Suisse expects to receive or intends to seek investment banking related compensation from the subject company (PTCT.OQ) within the next 3 months.

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As of the end of the preceding month, Credit Suisse beneficially own 1% or more of a class of common equity securities of (PTCT.OQ).

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