

#### **EARNINGS UPDATE**

#### **Biotechnology**

November 14, 2013

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#### Recommendation

Rating:	Outperform
Price Target (in \$):	\$33.00
Expected Return:	102.3%
Dividend:	NA
Enterprise Value (MM):	\$248.9

#### **Earnings Per Share**

	2012A	2013E	2014E
Q1		\$(2.08)A	\$(0.68)
Prev:			\$(0.67)
Q2		\$(5.51)A	\$(0.71)
Prev:			\$(0.70)
Q3		\$(0.19)A	\$(0.73)
Prev:		\$(0.21)	\$(0.72)
Q4		\$(0.59)	\$(0.75)
Prev:		\$(0.60)	\$(0.74)
FY	<u>\$219.76</u>	\$(4.04)	\$(2.86)
Prev:		\$(4.05)	\$(2.82)
P/E	0.1x	NM	NM

#### Stock Statistics as of 11/13/2013 (in \$)

Price:	\$16.31
52W Range:	\$24.38-\$13.04
Shares Out (MM):	24.9
Market Cap (MM):	\$406.1
Net Debt (MM):	\$0.0
Net Cash Per Share:	\$6.65

#### **Fundamentals**

Revenue (MM) ('12A)	33.9
Revenue (MM) ('13E)	36.8
Revenue (MM) ('14E)	19.0
EV/S ('12)	7.3x
EV/S ('13)	6.8x
EV/S ('14)	13.1x



# PTC THERAPEUTICS, INC. (NASDAQ:PTCT)

# PTC Platform Technology Creating A Wealth Of Value

This morning before the open, PTC announced 3Q13 financials. We view PTC as an attractive investment opportunity that combines multiple near-term catalysts with long-term growth potential. We remain bullish on PTC shares and reiterate our Outperform rating.

# Confirmatory Phase III clinical trial in nmDMD is on track to complete patient enrollment by mid-2014.

Top-line data are expected by mid-2015 and we remain optimistic that the trial will likely succeed based on the encouraging data from the Phase IIb clinical trial. In Europe, the CHMP will convene a Scientific Advisory Committee (SAC) meeting to facilitate active discussion among independent experts and patient advocacy groups. Based on the feedback from the SAC meeting, the final opinions from the CHMP regarding the conditional approval of ataluren in nmDMD is expected in 1Q14. PTC now plans to submit the MAA for ataluren conditional approval in nmCF in 1Q14 to prioritize regulatory pursuit for the nmDMD program. The second Phase III clinical trial for nmCF will be initiated in 1H14 and will have a similar design to the first Phase III study.

#### PTC is building a robust pipeline.

At the recent Analyst Day, management provided the Street with a comprehensive overview of the company's pipeline which is built on management's extensive expertise on post-transcriptional modulation and is focused on Orphan indications. The most promising program is in spinal muscular atrophy (SMA) and PTC received a \$10MM milestone payment in 3Q13 from partner Roche upon lead candidate selection.

#### PTC ended 3Q13 with a strong balance sheet.

PTC reported a loss per share of \$0.19 as compared to our estimate of a loss of \$0.21 and the consensus estimate of a loss of \$0.30. The cash balance at the end of 3Q13 was approximately \$157.2MM and should sustain the company's operations through the top-line data readout from the nmDMD confirmatory Phase III clinical trial, in our opinion.

Please see addendum of this report for important disclosures.

Exhibit 1. PTC Therapeutics, Inc. Upcoming Milestones

	Events	Time
	Potential conditional approval in the EU	1Q14
⅀	Completion of patient enrollment for the confirmatory Phase III clinical trial	Mid-2014
nmDMD	Top-line data from the confirmatory Phase III clinical trial	Mid-2015
	FDA and MAA filing for full approval	2H15/2016
	MAA filing for conditional approval in the EU	1Q14
ų,	MAA filing for conditional approval in the EU Initiation of confirmatory Phase III clinical trial	1Q14 1H14
mCF		•
nmCF	Initiation of confirmatory Phase III clinical trial	1H14
nmCF	Initiation of confirmatory Phase III clinical trial Potential conditional approval in the EU	1H14 2H14

Source: Cowen and Company

Exhibit 2. Robust Pipeline Based On Proprietary Technology Platform

Robust pipeline	<ul> <li>SMA alternative splicing program – partnered with Roche</li> <li>BMI1: cancer stem cell program – supported by Wellcome Trust</li> <li>Antibacterial program – supported by Wellcome Trust</li> </ul>
Platform technologies: Post transcriptional control	<ul> <li>Nonsense suppression</li> <li>Alternative splicing</li> <li>Nonsense mediated decay</li> <li>Protein modification</li> <li>Transcript regulators</li> </ul>

Source: PTC Therapeutics, Inc.

Exhibit 3. PTC Therapeutics, Inc. Quarterly P&L Model (\$MM)

	2011A	2012A	Q1:13A	Q2:13A	Q3:13A	Q4:13E	2013E	Q1:14E	Q2:14E	Q3:14E	Q4:14E	2014
Revenues												
Atularen product sales revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.
Collaboration revenue	99.0	28.8	6.1	5.9	15.5	5.6	33.0	3.8	3.8	3.8	3.8	15.
Grant revenue	6.5	5.2	1.1	1.0	0.8	0.9	3.8	10	1.0	1.0	1.0	4.0
Total revenues and non-cash cancellation revenue	105.4	33.9	7.1	6.9	16.3	6.5	36.8	4.8	4.8	4.8	4.8	19.0
Operating Expenses												
COGS	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.
Research and development	58.7	46.1	11.3	14.7	13.9	14.6	54.5	15.5	16.3	16.8	17.4	66.
General and administrative	16.2	14.6	4.5	6.6	6.7	6.8	24.5	6.5	6.7	6.8	7.0	27.0
Sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total Operating Expenses	74.8	60.8	15.7	21.3	20.6	21.4	79.0	22.0	23.0	23.6	24.4	93.0
Income (loss) from operating	30.6	(26.8)	(8.6)	(14.5)	(4.3)	(14.9)	(42.2)	(17.3)	(18.3)	(18.9)	(19.7)	(74.0
Other non-operating income (loss)												
Interest income (expense), net	(2.4)	(12)	(6.2)	(0.1)	0.0	0.1	(6.1)	0.0	0.0	0.0	0.0	0.
Loss on extinguishment of debt			` -		(0.1)	-		-	-	-	-	
Other income (expense), net	0.5	1.8	0.1	(0.0)	(0.0)	0.0	0.0	0.1	0.1	0.1	0.1	0.4
Income (loss) from operations before tax benefit	28.6	(26.2)	(14.7)	(14.6)	(4.4)	(14.7)	(48.3)	(17.2)	(18.2)	(18.8)	(19.6)	(73.6
Tax benefit	2.3	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Deemed dividend	0.0	0.0	(18.2)	0.0	0.0	0.0	(18.2)	0.0	0.0	0.0	0.0	0.0
Net Income (Loss)	30.9	(26.2)	(32.9)	(14.6)	(4.4)	(14.7)	(66.5)	(17.2)	(18.2)	(18.8)	(19.6)	(73.6
Gain on exchange of convertible perferred stock in connection with												
recapitalization	0.0	160.0	3.4	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Less beneficial conversion charge	0.0	(0.4)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net income attributable to common stockholders	30.9	133.3	(29.5)	(14.6)	(4.4)	(14.7)	(66.5)	(17.2)	(18.2)	(18.8)	(19.6)	(73.6
Taxrate	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Income Tax	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net income (loss) per share - basic	23.95	219.76	(2.08)	(5.51)	(0.19)	(0.59)	(4.04)	(0.68)	(0.71)	(0.73)	(0.75)	(2.86
Net income (loss) per share - diluted	4.55	42.50	(1.83)	(5.51)	(0.19)	(0.59)	(4.04)	(0.68)	(0.71)	(0.73)	(0.75)	(2.86
Weighted average common shares outstanding - basic	0.001	0.003	14.2	2.6	23.8	25.2	16.5	25.4	25.6	25.8	26.0	25.
Weighted average common shares outstanding - diluted	0.006	0.017	16.1	2.6	23.8	25.2	16.5	25.4	25.6	25.8	26.0	25.

Source: Cowen and Company

Exhibit 4. PTC Therapeutics, Inc. Annual P&L Model (\$MM)

	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025
Revenues															
Atularen product sales revenue	0.0	0.0	0.0	0.0	0.0	27.1	202.2	355.0	508.3	604.5	704.3	807.5	914.1	979.3	975
Collaboration revenue	99.0	28.8	33.0	15.0	10.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	(
Grant revenue	6.5	5.2	3.8	4.0	5.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
Total revenues and non-cash cancellation revenue	105.4	33.9	36.8	19.0	15.0	27.1	202.2	355.0	508.3	604.5	704.3	807.5	914.1	979.3	975
Operating Expenses															
COGS	0.0	0.0	0.0	0.0	0.0	4.1	28.3	46.2	610	66.5	70.4	80.7	914	97.9	9
Research and development	58.7	46.1	54.5	66.0	68.0	70.0	65.0	60.0	60.0	60.0	65.0	70.0	75.0	80.0	
General and administrative	16.2	14.6	24.5	27.0	30.0	35.0	37.0	40.0	43.0	45.0	48.0	50.0	52.0	55.0	
Sales	0.0	0.0	0.0	0.0	2.5	12.5	25.0	27.5	30.3	33.3	36.6	40.3	44.3	48.7	
Total Operating Expenses	74.8	60.8	79.0	93.0	100.5	121.6	155.3	173.7	194.2	204.8	220.0	241.0	262.7	281.7	29
Income (loss) from operating	30.6	(26.8)	(42.2)	(74.0)	(85.5)	(94.5)	46.9	181.4	314.1	399.8	484.2	566.5	651.4	697.7	682
Other non-operating income (loss)															
Interest income (expense), net	(2.4)	(1.2)	(6.1)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
Loss on extinguishment of debt				-		-	-	-				-			
Other income (expense), net	0.5	1.8	0.0	0.4	0.5	0.5	0.5	0.5	0.5	0.5	0.5	0.5	0.5	0.5	
ncome (loss) from operations before tax benefit	28.6	(26.2)	(48.3)	(73.6)	(85.0)	(94.0)	47.4	18 1.9	314.6	400.3	484.7	567.0	651.9	698.2	68:
Tax benefit	2.3	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
Deemed dividend	0.0	0.0	(18.2)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
Net Income (Loss)	30.9	(26.2)	(66.5)	(73.6)	(85.0)	(94.0)	47.4	18 1.9	314.6	400.3	484.7	567.0	651.9	698.2	682
Sain on exchange of convertible perferred stock in connection with															
recapitalization	0.0	160.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
_ess beneficial conversion charge	0.0	(0.4)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	
Net income attributable to common stockholders	30.9	133.3	(66.5)	(73.6)	(85.0)	(94.0)	47.4	18 1.9	314.6	400.3	484.7	567.0	651.9	698.2	68:
Tax rate	0%	0%	0%	0%	0%	0%	3%	8%	2%	18%	27%	35%	35%	35%	
ncome Tax	0.0	0.0	0.0	0.0	0.0	0.0	1.4	14.5	37.7	72.0	130.9	198.4	228.2	244.4	2
Net income (loss) per share - basic	23.95	219.76	(4.04)	(2.86)	(2.46)	(2.62)	1.11	3.97	6.41	7.29	7.78	7.96	8.98	9.45	8
Net income (loss) per share - diluted	4.55	42.50	(4.04)	(2.86)	(2.46)	(2.62)	1.06	3.80	6.14	7.00	7.47	7.65	8.63	9.09	8
Veighted average common shares outstanding - basic	0.001	0.003	16.5	25.7	34.5	35.8	41.3	42.1	43.2	45.0	45.5	46.3	47.2	48.0	
Weighted average common shares outstanding - diluted	0.006	0.017	16.5	25.7	34.5	35.8	43.2	44.0	45.1	46.9	47.4	48.2	49.1	49.9	

Source: Cowen and Company



## Valuation Methodology & Investment Risks

#### **Valuation Methodology**

#### Biotechnology:

In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

#### **Investment Risks**

#### Biotechnology:

There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

#### Company Specific Risks

The Phase III clinical trial for nmDMD and the Phase III clinical trial for nmCF that PTC completed failed to achieve the pre-specified primary endpoints with statistical significance. There is no guarantee that the ongoing and the planned Phase III clinical trials will meet the primary endpoint even though PTC has modified the trial designs to demonstrate maximum clinical benefit. Additionally, the EMA has raised questions about ataluren's insufficient efficacy and optimal dose and therefore, may reject PTC's application for conditional approval in the EU. As a result, even if the Phase III clinical trials succeed, ataluren will not be able to enter the market for several years. PTC's current balance sheet is strong but we estimate that there will be a need for additional funding to complete the trials for regulatory approval in the U.S.



### Addendum

#### STOCKS MENTIONED IN IMPORTANT DISCLOSURES

Ticker	Company Name
PTCT	PTC Therapeutics, Inc.

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Each author of this research report hereby certifies that (i) the views expressed in the research report accurately reflect his or her personal views about any and all of the subject securities or issuers, and (ii) no part of his or her compensation was, is, or will be related, directly or indirectly, to the specific recommendations or views expressed in this report.

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#### **COWEN AND COMPANY RATING DEFINITIONS**

#### Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

#### Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

#### Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

**Buy** – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

**Sell** – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

**Hold** – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

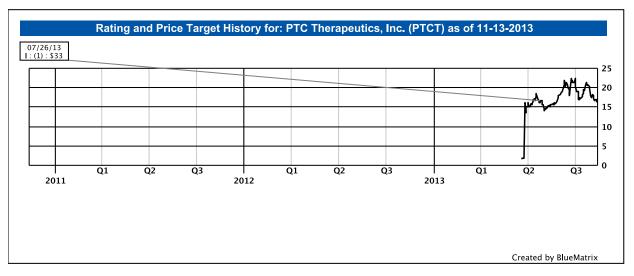
#### **COWEN AND COMPANY RATING ALLOCATION**

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	394	58.72%	54	13.71%
Hold (b)	255	38.00%	5	1.96%
Sell (c)	22	3.28%	1	4.55%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.



#### **Legend for Price Chart:**

I = Initation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | \$xx = Price Target | NA = Not Available