

## FLASH NOTE

### Biotechnology

November 13, 2013

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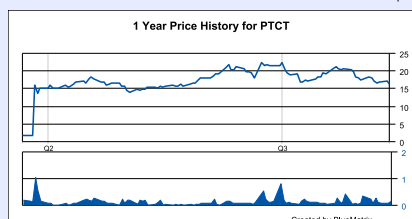
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### Recommendation

Rating:	Outperform
Price Target:	\$33.00

### Stock Statistics as of 11/12/2013

Price:	\$16.42
52W Range:	\$24.38-\$13.04
Shares Out (MM):	24.9
Market Cap (MM):	\$409.2
Net Debt (MM):	\$2.2
Net Cash Per Share:	\$6.65



## PTC THERAPEUTICS, INC. (NASDAQ:PTCT)

# For Sarepta It Is Wait And See, But In No Way Affects PTC

Yesterday Sarepta Therapeutics announced that the FDA considered an NDA filing for eteplirsen as premature and cited "previous negative reports for PTC124 (ataluren)" as part of the reason why the agency raised doubt about dystrophin as a reliable biomarker "to reasonably likely predict clinical benefit." Additionally, in response to Sarepta's claim that it would be difficult to enroll enough patient into a placebo-controlled confirmatory study, the FDA suggested using endpoints other than the six-minute walk test (6MWT). We believe the news is neutral to PTC and that in fact the FDA's comments on trial design and appropriate patient population support our investment thesis that PTC's ongoing Phase III clinical trial in nmDMD will likely succeed. We remain bullish on PTC shares and reiterate our Outperform rating.

### PTC's Phase III Clinical Trial design maximizes the possibility of demonstrating clinical benefit

The FDA commented that a baseline 6MWT of over 350 meters "predicts continued general stability" in DMD patients. This is the same conclusion reached by PTC's natural history studies and is also the basis of the patient inclusion criteria for the ongoing Phase III study. With 110 patients to be enrolled in each of the ataluren and placebo arms, the trial is using the 6MWT at 48 weeks as the primary endpoint. and is powered at 85% to detect an improvement of 30 meters. We believe the FDA recognizes the 6MWT as a widely used and well-established clinical endpoint since the agency stressed that it would accept "6MWT in an appropriately powered study." Therefore, we believe the trial is well designed and had a high likelihood of success given that a retrospective subgroup analysis of the Phase IIb patients who would qualify for the Phase III inclusion criteria demonstrated a clinical benefit of 49.9 meters with high statistical significance.

### Patient enrollment for the Phase III study is on track

PTC has guided that patient enrollment will be completed in mid-2014 and that top-line data will be available in mid-2015. The company is expecting a response from the EMA in 4Q13 regarding the MAA for conditional approval for ataluren in nmDMD, which we have not included in our company valuation and treat as additional upside potential.

Please see addendum of this report for important disclosures.



## 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 IIb 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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##### ***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

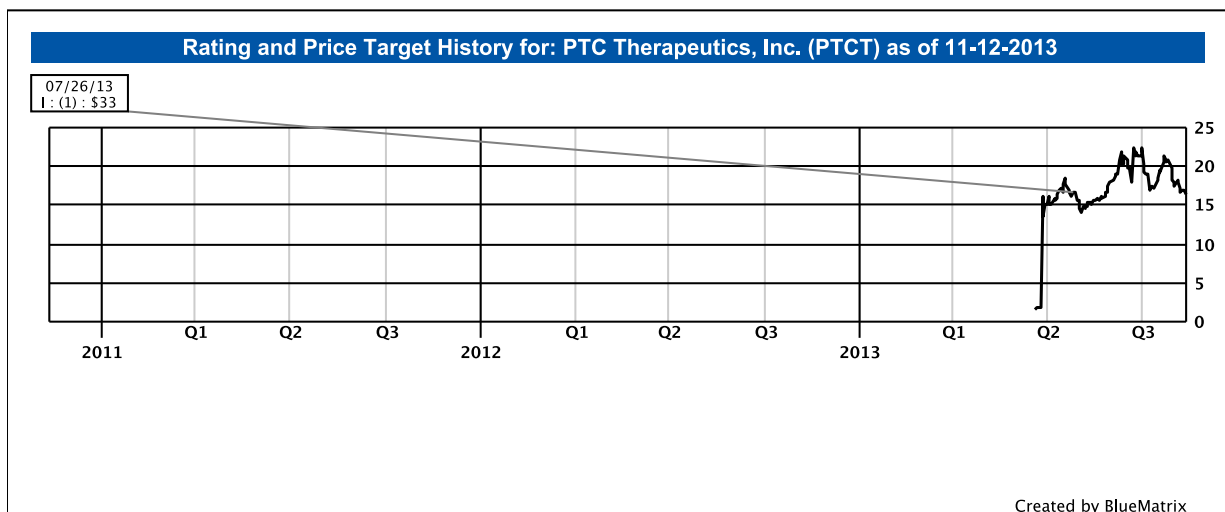
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##### ***Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13***

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.

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**Legend for Price Chart:**

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