

**FLASH NOTE**

**Biotechnology**

September 23, 2013

Edward Nash

[edward.nash@cowen.com](mailto:edward.nash@cowen.com) 646.562.1385

Yun Zhong, Ph.D.

[yun.zhong@cowen.com](mailto:yun.zhong@cowen.com) 646.562.1387

Joon Lee, M.D., Ph.D.

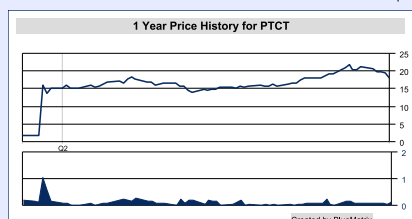
[joon.lee@cowen.com](mailto:joon.lee@cowen.com) 646.562.1326

**Recommendation**

Rating:	Outperform
Price Target:	\$33.00

**Stock Statistics as of 09/20/2013**

Price:	\$17.88
52W Range:	\$22.37-\$13.04
Shares Out (MM):	24.9
Market Cap (MM):	\$445.5
Net Debt (MM):	\$0.0
Net Cash Per Share:	\$6.65



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

# Ataluren Highly Differentiated and Miles Away from Friday's DMD Data Debacle

PTC's shares took a hard dip last Friday, and we believe the drop was directly related to the negative results that GSK/Prosensa reported from the Phase III clinical trial for drisapersen in DMD patients. Drisapersen failed to demonstrate a statistically significant improvement in the six-minute walking distance (6MWD) as compared to placebo after 48 weeks of treatment. Drisapersen is based on exon-skipping technology and targets DMD patients with genetic mutations in the *dystrophin* gene that are amenable to skipping of exon 51. In contrast, PTC's ataluren causes read-through of premature stop codons and target a non-overlapping different subpopulation of DMD patient. Since there is **neither similarity in mechanism of action nor direct competition between the two drugs**, we believe the data readout from drisapersen trial should be a non-event for PTC.

**Drisapersen trial confirms natural history data in DMD patients older than seven**

Prosensa's management commented at last Friday morning's investor call that the Phase III clinical trial demonstrated a decline in the 6MWD in patients over the age of seven that is in line with the 40m to 60m/year decline reported by natural history studies to date. This result is a positive for PTC, in our opinion, since the company's ongoing Phase III clinical trial is enrolling patients over the age of seven, and a larger decline in this patient population will help ataluren better demonstrate its efficacy in maintaining DMD patients' walking ability.

**Trial size difference is less of an issue for PTC**

The disappointing results from drisapersen's Phase III clinical trial reminded investors once again that successful Phase II trials do not necessarily guarantee positive Phase III results. However, we believe the risk of not being able to replicate Phase II data in Phase III trial is limited for PTC. Drisapersen's Phase II trial (DEMAND II) that met the primary endpoint enrolled only 18 patients in each of the active treatment arms (continuous dosing) and the placebo arm. The trial demonstrated a clinical benefit of approximately 35m. The Phase III trial enrolled 125 patients in the active treatment arm and 61 patients in the placebo arm. In contrast, ataluren's Phase IIb trial enrolled 57 patients in each of the active treatment arm (low dose) and placebo arm and demonstrated a clinical benefit of approximately 30m. The Phase III trial expects to enroll 110 patients in each arm. Moreover, PTC has modified enrollment criteria and the clinical benefit from the subgroup of patients from the Phase IIb trial who would meet the Phase III criteria was approximately 50m (p=0.0096). Therefore, we are optimistic that the likelihood is high for ataluren's Phase III trial to meet the primary endpoint.

Please see addendum of this report for important disclosures.



### Positive readout from Phase III will end the debate on ataluren's mechanism of action

The weakness of PTC shares may also be due to a commentary article published in a recent issue of the journal of *Nature Biotechnology* (Volume 31, Number 9) which summarized the debate over the mechanism of action of ataluren in inducing premature stop codon read-through. We continue to believe the *in vivo* data generated by both PTC and other independent investigators, which demonstrated full length protein production by ataluren treatment, should be the most convincing evidence. A statistically significant clinical benefit from the ongoing Phase III clinical trial will be the strongest rebuttal by PTC. We remain bullish on PTC shares and reiterate our Outperform rating.



## 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

### Analyst Certification

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.

### Important Disclosures

Cowen and Company, LLC and or its affiliates make a market in the stock of PTC Therapeutics, Inc. securities.

PTC Therapeutics, Inc. has been client(s) of Cowen and Company, LLC in the past 12 months.

PTC Therapeutics, Inc. is or was in the past 12 months a client of Cowen and Company, LLC; during the past 12 months, Cowen and Company, LLC provided IB services.

Cowen and Company, LLC and/or its affiliates received in the past 12 months compensation for investment banking services from PTC Therapeutics, Inc..

Cowen and Company, LLC and/or its affiliates managed or co-managed a public offering of PTC Therapeutics, Inc. within the past twelve months.

Cowen and Company, LLC compensates research analysts for activities and services intended to benefit the firm's investor clients. Individual compensation determinations for research analysts, including the author(s) of this report, are based on a variety of factors, including the overall profitability of the firm and the total revenue derived from all sources, including revenues from investment banking. Cowen and Company, LLC does not compensate research analysts based on specific investment banking transactions.

### Disclaimer

This research is for our clients only. Our research is disseminated primarily electronically and, in some cases, in printed form. Research distributed electronically is available simultaneously to all Cowen and Company, LLC clients. All published research can be obtained on the Firm's client website, <https://cowenlibrary.bluematrix.com/client/library.jsp>.

Further information on any of the above securities may be obtained from our offices. This report is published solely for information purposes, and is not to be construed as an offer to sell or the solicitation of an offer to buy any security in any state where such an offer or solicitation would be illegal. Other than disclosures relating to Cowen and Company, LLC, the information herein is based on sources we believe to be reliable but is not guaranteed by us and does not purport to be a complete statement or summary of the available data. Any opinions expressed herein are statements of our judgment on this date and are subject to change without notice.

For important disclosures regarding the companies that are the subject of this research report, please contact Compliance Department, Cowen and Company, LLC, 599 Lexington Avenue, 20th Floor, New York, NY 10022. In addition, the same important disclosures, with the exception of the valuation methods and risks, are available on the Firm's disclosure website at <https://cowen.bluematrix.com/sellside/Disclosures.action>.

**Price Targets:** Cowen and Company, LLC assigns price targets on all covered companies unless noted otherwise. The price target for an issuer's stock represents the value that the analyst reasonably expects the stock to reach over a performance period of twelve months. The price targets in this report should be considered in the context of all prior published Cowen and Company, LLC research reports (including the disclosures in any such report or on the Firm's disclosure website), which may or may not include price targets, as well as developments relating to the issuer, its industry and the financial markets. For price target valuation methodology and risks associated with the achievement of any given price target, please see the analyst's research report publishing such targets.

**Notice to UK Investors:** This publication is produced by Cowen and Company, LLC which is regulated in the United States by FINRA. It is to be communicated only to persons of a kind described in Articles 19 and 49 of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005. It must not be further transmitted to any other person without our consent.

### Copyright, User Agreement and other general information related to this report

© 2013 Cowen and Company, LLC. Member NYSE, FINRA and SIPC. All rights reserved. This research report is prepared for the exclusive use of Cowen clients and may not be reproduced, displayed, modified, distributed, transmitted or disclosed, in whole or in part, or in any form or manner, to others outside your organization without the express prior written consent of Cowen. Cowen research reports are distributed simultaneously to all clients eligible to receive such research reports. Any unauthorized use or disclosure is prohibited. Receipt and/or review of this research constitutes your agreement not to reproduce, display, modify, distribute, transmit, or disclose to others outside your organization the contents, opinions, conclusion,



or information contained in this report (including any investment recommendations, estimates or price targets). All Cowen trademarks displayed in this report are owned by Cowen and may not be used without its prior written consent.

**Cowen and Company, LLC.** New York (646) 562-1000 **Boston** (617) 946-3700 **San Francisco** (415) 646-7200 **Chicago** (312) 577-2240 **Cleveland** (440) 331-3531 **Atlanta** (866) 544-7009 **London** (affiliate) 44-207-071-7500

## **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

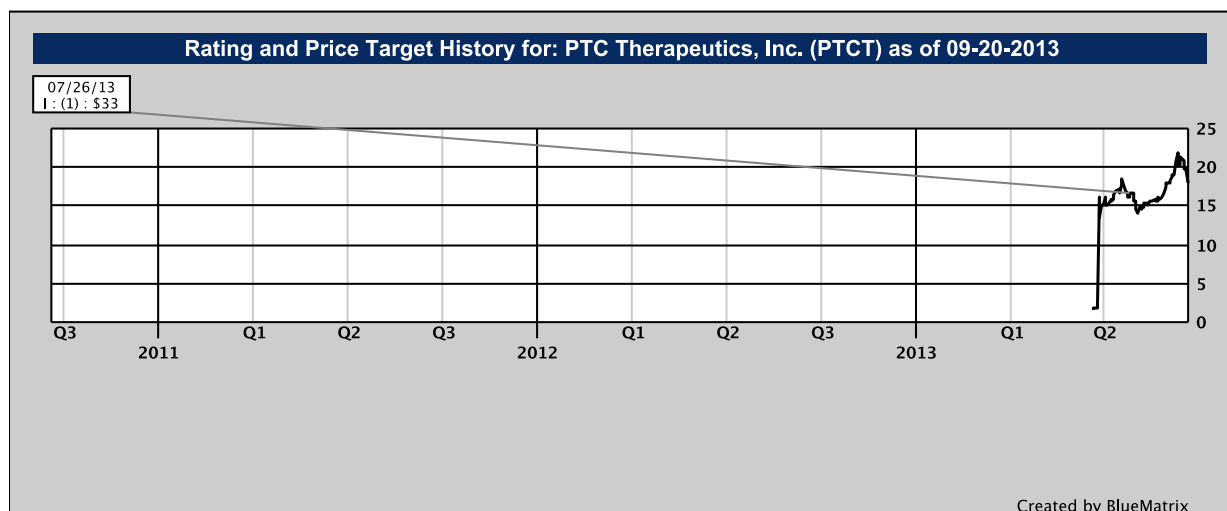
## **COWEN AND COMPANY RATING ALLOCATION**

### ***Distribution of Ratings/Investment Banking Services (IB) as of 06/30/13***

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	380	58.37%	48	12.63%
Hold (b)	247	37.94%	2	0.81%
Sell (c)	24	3.68%	1	4.17%

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