

Equity Research

January 24, 2014

Price: \$26.22 (01/23/2014)

Price Target: \$33.00

OUTPERFORM (1)

Edward Nash

646.562.1385
edward.nash@cowen.com

Yun Zhong, Ph.D.

646.562.1387
yun.zhong@cowen.com

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

646.562.1326
joon.lee@cowen.com

Key Data

Symbol [NASDAQ: PTCT](#)
Market Cap (MM) [\\$653.3](#)

Company Quick Take

Spring Cleaning Comes Early To PTC

The Cowen Insight

This morning, PTC announced that the CHMP has adopted a negative opinion on PTC's MAA for Conditional Approval of ataluren for nmDMD. This outcome is consistent with Management's expectation and guidance to date. We never included a Conditional Approval in our model so our valuation is not affected by the news. We reiterate our Outperform rating on PTC shares and our 12-month price target of \$33.

Positive data from the ongoing Phase III clinical trial will be the real value driver

Management commented that a major concern from the CHMP was the potential impact of a Conditional Approval on the enrollment of the ongoing Phase III confirmatory study. PTC initiated the study in April 2013 and patient enrollment is on track to be completed in mid-2014. PTC intends to request a re-examination of the CHMP opinion with a final decision to be expected in 2Q14, when the confirmatory study is more fully enrolled.

We believe the negative opinion from the CHMP in no way changes the fundamentals of our PTC investment thesis. Management had always cautioned investors about the risk of a negative opinion and has been working diligently in initiating the confirmatory Phase III study, which is expected to report top-line data in mid-2015. The MAA submission for conditional approval was based on a meaningful clinical benefit of approximately 30 meters observed in the completed Phase IIb clinical trial. Information from the Phase IIb study has helped PTC identify the optimal group of patients to enroll in the ongoing Phase III. A retrospective analysis of the Phase IIb patients who would qualify for the inclusion criteria of the ongoing Phase III confirmatory study demonstrated an even higher clinical benefit of 49.9 meters with strong statistical significance. The Phase III study is powered at 85% to detect a clinical benefit of 30 meters. Therefore, we are optimistic that the top-line data from the study will be positive.

PTC has stated that the decision to submit an MAA for conditional approval of ataluren for the treatment of nmCF will be dependent of the CHMP's opinion on the conditional approval for nmDMD. Again, we have not included a conditional approval for nmCF in our model and our valuation is based on the assumption that the company will conduct a confirmatory Phase III clinical trial to support full approvals in both the U.S. and the EU.

We believe the drop in stock price in response to this morning's negative news has created a good buying opportunity for investors. Ataluren is the only therapeutic in clinical development for the treatment of nmDMD and nmCF and there is no overlap in targeted patient populations with other therapeutics being developed for DMD such as those based on exon-skipping technologies.

PTC also announced this morning that SVP of Clinical Development, Jay Barth, M.D. is leaving the company to pursue other opportunities. It was also announced that Robert Spiegel, M.D. will move from being interim Chief Medical Officer to full-time CMO. We view this as typical management housekeeping and in no way affects our fundamental analysis or valuation of PTC.

Anticipating continued timely clinical progress and a positive outcome from the ongoing Phase III clinical trial, we would be buyers of PTCT at current levels.

Valuation Methodology And 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.

Risks To The Price Target

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

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 securities.

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

PTC Therapeutics 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.

Cowen and Company, LLC and/or its affiliates managed or co-managed a public offering of PTC Therapeutics 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

© 2014 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 Dahlgren 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 Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	415	59.20%	68	16.39%
Hold (b)	270	38.52%	4	1.48%
Sell (c)	16	2.28%	1	6.25%

(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.

PTC Therapeutics Rating History as of 01/23/2014

powered by: BlueMatrix



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

Points Of Contact

Reaching Cowen

Main U.S. Locations

New York

599 Lexington Avenue
New York, NY 10022
646.562.1000
800.221.5616

Atlanta

3399 Peachtree Road NE
Suite 417
Atlanta, GA 30326
866.544.7009

Boston

Two International Place
Boston, MA 02110
617.946.3700
800.343.7068

Cleveland

20006 Detroit Road
Suite 100
Rocky River, OH 44116
440.331.3531

San Francisco

555 California Street, 5th Floor
San Francisco, CA 94104
415.646.7200
800.858.9316

Chicago

181 West Madison Street
Suite 1925
Chicago, IL 60602
312.577.2240

International Locations

Cowen International Limited**London**

1 Snowden Street - 11th Floor
London EC2A 2DQ
United Kingdom
44.20.7071.7500

Cowen and Company (Asia) Limited**Hong Kong**

Suite 1401 Henley Building
No. 5 Queens Road Central
Central, Hong Kong
852 3752 2333

