EARNINGS UPDATE
Biotechnology
August 13, 2013

Edward Nash
edward.nash@cowen.com 1 (646) 562-1385
Yun Zhong, Ph.D.
yun.zhong@cowen.com 1 (646) 562-1387
Joon Lee, M.D., Ph.D.
joon.lee@cowen.com 1 (646) 562-1326

# PTC THERAPEUTICS, INC. (NASDAQ:PTCT)

# 2Q13: Quarterly Call Debut Confirms All Milestones on Track

**Yesterday, after the close, PTC reported 2Q13 financials and conducted the first investor call since the company successfully completed the IPO in June 2013. With all clinical programs progressing as planned, we remain bullish on PTC shares and reiterate our Outperform rating.**

**Recommendation**

| | |
|---|---|
| Rating: | Outperform |
| Price Target (in $): | $33.00 |
| Expected Return: | 113.5% |
| Dividend: | NA |
| Enterprise Value (MM): | $219.6 |

**Earnings Per Share**

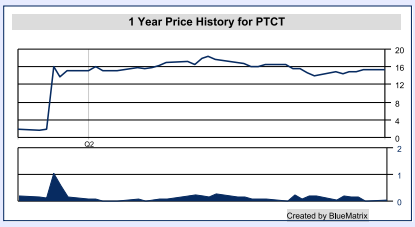
| | 2012A | 2013E | 2014E |
|---|---|---|---|
| **Q1** | -- | *$(2.08)A* | *$(0.67)* |
| *Prev:* | -- | -- | *$(0.56)* |
| **Q2** | -- | *$(5.51)A* | *$(0.70)* |
| *Prev:* | -- | *$(0.77)* | *$(0.61)* |
| **Q3** | -- | *$(0.21)* | *$(0.72)* |
| *Prev:* | -- | *$(0.44)* | *$(0.66)* |
| **Q4** | -- | *$(0.60)* | *$(0.74)* |
| *Prev:* | -- | *$(0.06)* | *$(0.68)* |
| **FY** | $219.76 | *$(4.05)* | *$(2.82)* |
| *Prev:* | -- | *$(2.88)* | *$(2.51)* |
| **P/E** | 0.1x | NM | NM |

**Stock Statistics as of 08/12/2013 (in $)**

| | |
|---|---|
| Price: | $15.46 |
| 52W Range: | $18.50-$13.04 |
| Shares Out (MM): | 24.9 |
| Market Cap (MM): | $385.2 |
| Net Cash Per Share: | $6.65 |

**Fundamentals**

| | |
|---|---|
| Revenue (MM) ('12A) | 33.9 |
| Revenue (MM) ('13E) | 37.5 |
| Revenue (MM) ('14E) | 20.0 |
| EV/S ('12) | 6.5x |
| EV/S ('13) | 5.9x |
| EV/S ('14) | 11.0x |




## A strong balance sheet will support sustained growth as a public company.

PTC reported a loss per share of $5.51 as compared to our estimate and the consensus of a loss of $0.77, due to the number of shares calculated related to the IPO. The company ended 2Q13 with approximately $165.7MM in cash, cash equivalents, and marketable securities. We believe the strong balance sheet will sustain the company's operations through top-line data readout from the ongoing confirmatory Phase III nmDMD trial, which is expected in mid-2015. Patient enrollment will complete by mid-2014, and we remain confident that the inclusion criteria are highly favorable for ataluren to demonstrate a statistically significant improvement in clinical benefit as compared to placebo. On the nmCF end, patient enrollment for the confirmatory Phase III clinical trial will begin in 1H14.

## PTC expects multiple near-term clinical development milestones.

PTC submitted the responses to the Day 120 Questions in July 2013 to support the MAA for conditional approval of ataluren for nmDMD in the EU. A response from the CHMP is anticipated by year-end 2013. The company also plans to submit a similar MAA for conditional approval for nmCF by year-end 2013. Any positive feedback from the EMA on the MAAs should be a strong catalyst for PTC shares.

## SMA program is ready to move forward.

Last week, PTC announced the selection of the lead development candidate for the SMA program, for which PTC was entitled to a $10MM milestone payment from Roche. We believe SMA represents a significant opportunity for PTC, given the severity of this Orphan indication and the lack of effective treatments. We expect PTC and Roche to initiate clinical development rapidly, and we believe the preclinical data are highly promising.

Please see addendum of this report for important disclosures.

## Both nmDMD and nmCF Confirmatory Phase III Clinical Trials Are De-Risked

PTC has completed one Phase IIb clinical trial for nmDMD and one Phase III clinical trial for nmCF. Although both trials failed to achieve statistical significance in respective pre-specified primary endpoints, results from the two trials demonstrated promising trends of clinical benefit from treatment. A *post hoc* analysis of the nmDMD trial data demonstrated a trend towards statistical significance with the p value reaching 0.0561. Additionally, a subgroup analysis of the nmCF trial data demonstrated a much improved clinical benefit with the p value going from 0.0478 to 0.008. More importantly, PTC has identified the optimal patient populations for both indications and has designed the Phase III clinical trials accordingly to demonstrate maximum clinical benefit. Therefore, we are confident that both trials will deliver positive outcomes.

Ataluren is the only drug candidate currently in clinical development for nmDMD and nmCF patients. Our financial models, which are based on the nmDMD and the nmCF programs alone without a conditional approval in the EU for either program, suggests that ataluren can address a combined market of nearly $1 billion and that PTC shares are undervalued at the current level. Ataluren's activity in suppressing nonsense mutations can be applied to additional eligible genetic disorders and PTC has technology platforms that target other large unmet medical needs. Therefore, we believe there is significant upside potential and that PTC represents an attractive investment opportunity.

**Exhibit 1. Upcoming Milestones**

| | Events | Time |
|---|---|---|
| nmDMD | Potential conditional approval in the EU | 2H13 |
| | Completion of patient enrollment for the confirmatory Phase III clinical trial | Mid-2014 |
| | Top-line data from the confirmatory Phase III clinical trial | Mid-2015 |
| | FDA and MAA filing for full approval | 2H15 |
| nmCF | MAA filing for conditional approval in the EU | 2H13 |
| | Initiation of confirmatory Phase III clinical trial | 1H14 |
| | Potential conditional approval in the EU | 2H14 |
| | Completion of patient enrollment for the confirmatory Phase III clinical trial | 2H15 |

Source: PTC Therapeutics, Inc. & Cowen and Company

## Exhibit 2. PTC Therapeutics, Inc. Quarterly P&L Model ($MM)

| | 2011A | 2012A | Q1'13A | Q2'13A | Q3'13E | Q4'13E | 2013E | Q1'14E | Q2'14E | Q3'14E | Q4'14E | 2014E |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **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 | 0.0 |
| Collaboration revenue | 99.0 | 28.8 | 6.1 | 5.9 | 15.5 | 5.6 | 33.0 | 3.8 | 3.8 | 3.8 | 3.8 | 15.0 |
| Grant revenue | 6.5 | 5.2 | 1.1 | 1.0 | 1.1 | 1.3 | 4.5 | 1.3 | 1.3 | 1.3 | 1.3 | 5.0 |
| **Total revenues and non-cash cancellation revenue** | **105.4** | **33.9** | **7.1** | **6.9** | **16.6** | **6.9** | **37.5** | **5.0** | **5.0** | **5.0** | **5.0** | **20.0** |
| **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 | 0.0 |
| Research and development | 58.7 | 46.1 | 11.3 | 14.7 | 15.1 | 15.4 | 56.5 | 15.5 | 16.3 | 16.8 | 17.4 | 66.0 |
| General and administrative | 16.2 | 14.6 | 4.5 | 6.6 | 6.7 | 6.7 | 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** | **21.8** | **22.2** | **81.0** | **22.0** | **23.0** | **23.6** | **24.4** | **93.0** |
| **Income (loss) from operating** | **30.6** | **(26.8)** | **(8.6)** | **(14.5)** | **(5.2)** | **(15.3)** | **(43.5)** | **(17.0)** | **(18.0)** | **(18.6)** | **(19.4)** | **(73.0)** |
| **Other non-operating income (loss)** | | | | | | | | | | | | |
| Interest income (expense), net | (2.4) | (12) | (6.2) | (0.1) | (0.1) | (0.1) | (6.5) | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Other income (expense), net | 0.5 | 1.8 | 0.1 | (0.0) | 0.1 | 0.3 | 0.4 | 0.1 | 0.1 | 0.1 | 0.2 | 0.5 |
| **Income (loss) from operations before tax benefit** | **28.6** | **(26.2)** | **(14.7)** | **(14.6)** | **(5.2)** | **(15.1)** | **(49.6)** | **(16.9)** | **(17.9)** | **(18.5)** | **(19.2)** | **(72.5)** |
| 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 |
| Gain in exchange of convertible preferred stock in connection with recapitalization | | | | | | | | | | | | |
| Less beneficial conversion charge | | | | | | | | | | | | |
| **Net Income (Loss)** | **30.9** | **(26.2)** | **(32.9)** | **(14.6)** | **(5.2)** | **(15.1)** | **(67.8)** | **(16.9)** | **(17.9)** | **(18.5)** | **(19.2)** | **(72.5)** |
| 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)** | **(5.2)** | **(15.1)** | **(67.8)** | **(16.9)** | **(17.9)** | **(18.5)** | **(19.2)** | **(72.5)** |
| *Tax rate* | *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)** | **(0.21)** | **(0.60)** | **(4.05)** | **(0.67)** | **(0.70)** | **(0.72)** | **(0.74)** | **(2.82)** |
| **Net income (loss) per share - diluted** | **4.55** | **42.50** | **(1.83)** | **(5.51)** | **(0.21)** | **(0.60)** | **(4.05)** | **(0.67)** | **(0.70)** | **(0.72)** | **(0.74)** | **(2.82)** |
| Weighted average common shares outstanding - basic | 0.001 | 0.003 | 14.2 | 2.6 | 25.0 | 25.2 | 16.8 | 25.4 | 25.6 | 25.8 | 26.0 | 25.7 |
| Weighted average common shares outstanding - diluted | 0.006 | 0.017 | 16.1 | 2.6 | 25.0 | 25.2 | 16.8 | 25.4 | 25.6 | 25.8 | 26.0 | 25.7 |

Source: Cowen and Company

## Exhibit 3. PTC Therapeutics, Inc. Annual P&L Model ($MM)

| | 2011A | 2012A | 2013E | 2014E | 2015E | 2016E | 2017E | 2018E | 2019E | 2020E | 2021E | 2022E | 2023E | 2024E | 2025E |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **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 | 975.2 |
| 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 | 0.0 |
| Grant revenue | 6.5 | 5.2 | 4.5 | 5.0 | 5.0 | 0.0 | 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** | **37.5** | **20.0** | **15.0** | **27.1** | **202.2** | **355.0** | **508.3** | **604.5** | **704.3** | **807.5** | **914.1** | **979.3** | **975.2** |
| **Operating Expenses** | | | | | | | | | | | | | | | |
| COGS | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 4.1 | 28.3 | 46.2 | 61.0 | 66.5 | 70.4 | 80.7 | 91.4 | 97.9 | 97.5 |
| Research and development | 58.7 | 46.1 | 56.5 | 66.0 | 68.0 | 70.0 | 65.0 | 60.0 | 60.0 | 60.0 | 65.0 | 70.0 | 75.0 | 80.0 | 85.0 |
| 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 | 57.0 |
| 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 | 53.6 |
| **Total Operating Expenses** | **74.8** | **60.8** | **81.0** | **93.0** | **100.5** | **121.6** | **155.3** | **173.7** | **194.2** | **204.8** | **220.0** | **241.0** | **262.7** | **281.7** | **293.1** |
| **Income (loss) from operating** | **30.6** | **(26.8)** | **(43.5)** | **(73.0)** | **(85.5)** | **(94.5)** | **46.9** | **181.4** | **314.1** | **399.8** | **484.2** | **566.5** | **651.4** | **697.7** | **682.1** |
| **Other non-operating income (loss)** | | | | | | | | | | | | | | | |
| Interest income (expense), net | (2.4) | (12) | (6.5) | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Other income (expense), net | 0.5 | 1.8 | 0.4 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 | 0.5 |
| **Income (loss) from operations before tax benefit** | **28.6** | **(26.2)** | **(49.6)** | **(72.5)** | **(85.0)** | **(94.0)** | **47.4** | **181.9** | **314.6** | **400.3** | **484.7** | **567.0** | **651.9** | **698.2** | **682.6** |
| 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 | 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 | 0.0 |
| Gain in exchange of convertible preferred stock in connection with recapitalization | | | | | | | | | | | | | | | |
| Less beneficial conversion charge | | | | | | | | | | | | | | | |
| **Net Income (Loss)** | **30.9** | **(26.2)** | **(67.8)** | **(72.5)** | **(85.0)** | **(94.0)** | **47.4** | **181.9** | **314.6** | **400.3** | **484.7** | **567.0** | **651.9** | **698.2** | **682.6** |
| Gain 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 | 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 | 0.0 | 0.0 | 0.0 |
| **Net income attributable to common stockholders** | **30.9** | **133.3** | **(67.8)** | **(72.5)** | **(85.0)** | **(94.0)** | **47.4** | **181.9** | **314.6** | **400.3** | **484.7** | **567.0** | **651.9** | **698.2** | **682.6** |
| *Tax rate* | *0%* | *0%* | *0%* | *0%* | *0%* | *0%* | *3%* | *8%* | *12%* | *18%* | *27%* | *35%* | *35%* | *35%* | *35%* |
| Income 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 | 238.9 |
| **Net income (loss) per share - basic** | **23.95** | **219.76** | **(4.05)** | **(2.82)** | **(2.46)** | **(2.62)** | **1.11** | **3.97** | **6.41** | **7.29** | **7.78** | **7.96** | **8.98** | **9.45** | **8.96** |
| **Net income (loss) per share - diluted** | **4.55** | **42.50** | **(4.05)** | **(2.82)** | **(2.46)** | **(2.62)** | **1.06** | **3.80** | **6.14** | **7.00** | **7.47** | **7.65** | **8.63** | **9.09** | **8.63** |
| Weighted average common shares outstanding - basic | 0.001 | 0.003 | 16.8 | 25.7 | 34.5 | 35.8 | 41.3 | 42.1 | 43.2 | 45.0 | 45.5 | 46.3 | 47.2 | 48.0 | 49.5 |
| Weighted average common shares outstanding - diluted | 0.006 | 0.017 | 16.8 | 25.7 | 34.5 | 35.8 | 43.2 | 44.0 | 45.1 | 46.9 | 47.4 | 48.2 | 49.1 | 49.9 | 51.4 |

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 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

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**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

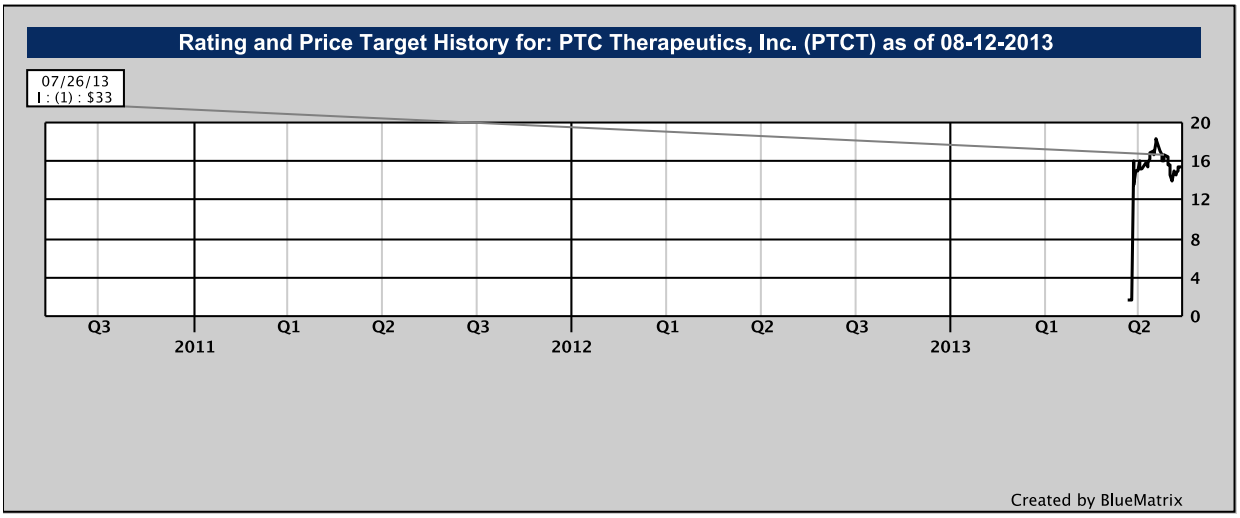
## COWEN AND COMPANY RATING ALLOCATION

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