COWEN
AND COMPANY

Biotechnology

PTC Therapeutics

Equity Research

Earnings Update

March 7, 2014

Price: $33.38 (03/6/2014)

Price Target: $40.00

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: PTCT
52-Week Range:	$34.65 - 13.04
Market Cap (MM):	$1,004.0
Net Debt (MM):	$0.0
Cash/Share:	$8.67
Dil. Shares Out (MM):	30.1
Enterprise Value (MM):	$743.9
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$6.35
FCF Yield:	NA
Dividend:	NA

FY (Dec)	2012A	2013E	2014E
Earnings Per Share			
Q1	-	$(2.08)A	$(0.38)
Q2	-	$(5.51)A	$(0.65)
Q3	-	$(0.19)A	$(0.65)
Q4	-	$(0.75)A	$(0.65)
Year	$219.76	$(5.18)A	$(2.34)
P/E	0.2x	NM	NM
Consensus EPS	-	$(3.78)	$(2.30)

Consensus source: Thomson Reuters

Revenue (MM)			
Q1	-	$7.1A	$12.3
Q2	-	$6.9A	$4.8
Q3	-	$16.3A	$4.8
Q4	-	$4.4A	$4.7
Year	$33.9	$34.7	$26.5

DMD, CF, SMA And Another Proof-Of-Concept Program Is On Its Way

The Cowen Insight

Yesterday after the close, PTC reported 4Q13 and FY13 financials. The company is making steady progress with regard to ataluren clinical development and we continue to be optimistic that the ongoing Phase III confirmatory study for nmDMD will be successful. We reiterate our Outperform rating on PTC shares and raise our 12-month price target from $33 to $40 based on the change in discount period..

Phase III Ataluren Confirmatory Trial in DMD (ACT DMD) study is on track

Patient enrollment will be completed by mid-2014 with top-line data expected in mid-2015, as PTC has guided. PTC has requested a re-examination of the negative opinion that CHMP adopted in January 2014 on the conditional approval of ataluren and expects to receive the final decision in 2Q14. However, our model and valuation are based on ataluren receiving full approval in both the U.S. and the EU in 2016. Our original price target of $33 was established at PTC's IPO in June 2013 and the change in our price target is solely due to the eight-month difference in the discount period and we have not adjusted any of our previous estimates.

Additional programs are progressing as well

PTC plans to initiate a Phase III confirmatory clinical trial of ataluren for nmCF in 1H14. The design will be similar to that of the completed Phase III study but Tobi use will not be allowed to minimize the negative impact of tobramycin on ataluren's nonsense readthrough activity. PTC will likely also apply for conditional approval for nmCF in the EU but our model does not include that scenario either. Management disclosed the plan to initiate proof-of-concept (POC) studies for at least one additional indication for ataluren in 2014. The choice of the indication will be based on whether patients are genetically well-defined, the availability of outcome measures for rapid POC validation, and preclinical data. Numerous studies have demonstrated ataluren's activity in animal models of multiple monogenic disorders. Therefore, we believe ataluren has significant potential as an orally available small molecule with a demonstrated strong safety profile. The SMA program in collaboration with Roche entered a Phase I study in healthy volunteers in January 2014 and a natural history study and an observational study are ongoing in SMA patients.

A strong balance sheet provides solid support for clinical success

PTC reported a loss per share of $0.75 as compared to our estimate and the consensus of a loss of $0.59 and $0.57, respectively. The larger loss was due to higher operational expenses in the quarter. The cash balance as of year-end 2013 was $142.5MM and PTC further completed a financing in February 2014 with net proceeds of approximately $118MM. We believe the strong balance sheet will sustain the company's operations through regulatory approval for ataluren for nmDMD in 2016.

At A Glance

Our Investment Thesis

Ataluren is the only drug candidate currently in clinical development for nonsense mutation Duchenne Muscular Dystrophy (nmDMD) and nonsense mutation cystic fibrosis (nmCF) patients. PTC has identified the optimal patient populations for both indications and has designed the Phase III confirmatory trials accordingly to demonstrate maximum clinical benefit. Therefore, we are confident that both confirmatory trials will deliver positive outcomes. Our financial model, which is 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 approximately $1 billion. 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.

Forthcoming Catalysts

- Patient enrollment completion in the ongoing Phase III confirmatory clinical trial for nmDMD in mid-2014
- Initiation of the Phase III confirmatory clinical trial for nmCF in 1H14
- Initiation of proof-of-concept studies of ataluren for additional indication(s) in 2014

Base Case Assumptions

- Ataluren receives FDA and EMA approvals for both nmDMD and nmCF
- PTC is able to build a proprietary sales force to market ataluren in both the U.S. and the EU
- PTC does not receive conditional approval for either indication in the EU

Upside Scenario

- PTC receives conditional approval(s) for ataluren for nmDMD and/or nmCF
- Ataluren achieves higher than expected market penetration
- PTC's additional clinical programs generate meaningful revenues for the company

Downside Scenario

- The Phase III confirmatory studies for nmDMD and/or nmCF fail to meet the primary endpoints
- PTC fails to commercialize ataluren efficiently in the U.S. or the EU

Price Performance

Source: Bloomberg

Company Description

PTC Therapeutics is developing orally available small molecule compounds for the treatment of genetic disorders by modulating post-transcriptional control processes. The company's lead drug candidate, ataluren, corrects nonsense mutations, which produce premature stop codons and disrupt proper protein production. Ataluren is in Phase III clinical development for nmDMD and nmCF. 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. 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 improving from 0.0478 to 0.008.

Analyst Top Picks

	Ticker	Price (03/6/2014)	Price Target	Rating
Regado Biosciences Inc	RGDO	$7.28	$14.00	Outperform
Ocera Therapeutics Inc	OCRX	$16.45	$NA	Outperform
Cempra	CEMP	$11.11	$15.00	Outperform

PTC Therapeutics Quarterly P&L Model ($MM)

	2011A	2012A	Q1:13A	Q2:13A	Q3:13A	Q4:13A	2013A	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	3.9	31.3	11.3	3.8	3.8	3.7	22.5
Grant revenue	6.5	5.2	1.1	1.0	0.8	0.5	3.4	1.0	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**	**4.4**	**34.7**	**12.3**	**4.8**	**4.8**	**4.7**	**$26.5**
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	13.9	15.0	54.9	15.8	16.8	17.5	17.9	68.0
General and Administrative	16.2	14.6	4.5	6.6	6.7	7.5	25.2	7.7	8.0	8.1	8.2	32.0
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**	**22.5**	**80.1**	**23.5**	**24.8**	**25.6**	**26.1**	**100.0**
Income (Loss) from Operations	**30.6**	**(26.8)**	**(8.6)**	**(14.5)**	**(4.3)**	**(18.1)**	**(45.4)**	**(11.2)**	**(20.1)**	**(20.9)**	**(21.4)**	**(73.5)**
Other non-operating income (loss)												
Interest income (expense), net	(2.4)	(1.2)	(6.2)	(0.1)	0.0	0.2	(6.1)	0.0	0.0	0.0	0.0	0.0
Loss on extinguishment of debt	-	-	-	-	(0.1)	-	(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)**	**(17.9)**	**(51.6)**	**(11.1)**	**(20.0)**	**(20.8)**	**(21.3)**	**(73.1)**
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)**	**(17.9)**	**(69.8)**	**(11.1)**	**(20.0)**	**(20.8)**	**(21.3)**	**(73.1)**
Gain on exchange of convertible perferred stock in connection with recapitalization	0.0	160.0	3.4	0.0	0.0	0.0	3.4	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)**	**(17.9)**	**(66.4)**	**(11.1)**	**(20.0)**	**(20.8)**	**(21.3)**	**(73.1)**
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)**	**(0.19)**	**(0.75)**	**(5.18)**	**(0.38)**	**(0.65)**	**(0.65)**	**(0.65)**	**($2.34)**
Net Income (Loss) per Share - Diluted	**4.55**	**42.50**	**(1.83)**	**(5.51)**	**(0.19)**	**(0.75)**	**(5.18)**	**(0.38)**	**(0.65)**	**(0.65)**	**(0.65)**	**($2.34)**
Weighted average common shares outstanding - basic	0.001	0.003	14.2	2.6	23.8	23.8	12.8	29.5	30.6	31.8	33.0	31.2
Weighted average common shares outstanding - diluted	0.006	0.017	16.1	2.6	23.8	23.8	12.8	29.5	30.6	31.8	33.0	31.2

Source: Cowen and Company

PTC Therapeutics Annual P&L Model ($MM)

	2011A	2012A	2013A	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	31.3	22.5	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	3.4	4.0	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**	**34.7**	**$26.5**	**$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	54.9	68.0	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	25.2	32.0	34.0	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**	**80.1**	**100.0**	**104.5**	**121.6**	**155.3**	**173.7**	**194.2**	**204.8**	**220.0**	**241.0**	**262.7**	**281.7**	**293.1**
Income (Loss) from Operations	**30.6**	**(26.8)**	**(45.4)**	**(73.5)**	**(89.5)**	**(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)	(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	0.0
Loss on extinguishment of debt	-	-	(0.1)	-	-	-	-	-	-	-	-	-	-	-	-
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	0.5
Income (loss) from operations before tax benefit	**28.6**	**(26.2)**	**(51.6)**	**(73.1)**	**(89.0)**	**(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
Net Income (Loss)	**30.9**	**(26.2)**	**(69.8)**	**(73.1)**	**(89.0)**	**(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	3.4	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
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**	**(66.4)**	**(73.1)**	**(89.0)**	**(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**	**(5.18)**	**($2.34)**	**($2.58)**	**($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**	**(5.18)**	**($2.34)**	**($2.58)**	**($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	12.8	31.2	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	12.8	31.2	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 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 endpoints even though PTC has modified the trial designs to demonstrate maximum clinical benefit. Our model is based on PTC marketing ataluren independently in both the U.S. and the EU. Therefore, if the company fails to execute the commercialization plan, ataluren will not be able to achieve the market potential which we believe the product is entitled to.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
CEMP	Cempra
OCRX	Ocera Therapeutics Inc
PTCT	PTC Therapeutics
RGDO	Regado Biosciences Inc

Analyst Certification

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Important Disclosures

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

PTC Therapeutics, Cempra, Ocera Therapeutics Inc and Regado Biosciences Inc have been client(s) of Cowen and Company, LLC in the past 12 months.

Cowen and Company, LLC and/or its affiliates expect to receive, or intend to seek, compensation for investment banking services in the next 3 months from PTC Therapeutics, Cempra and Regado Biosciences Inc.

PTC Therapeutics, Cempra, Ocera Therapeutics Inc and Regado Biosciences 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, Cempra, Ocera Therapeutics Inc and Regado Biosciences Inc.

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

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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.

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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

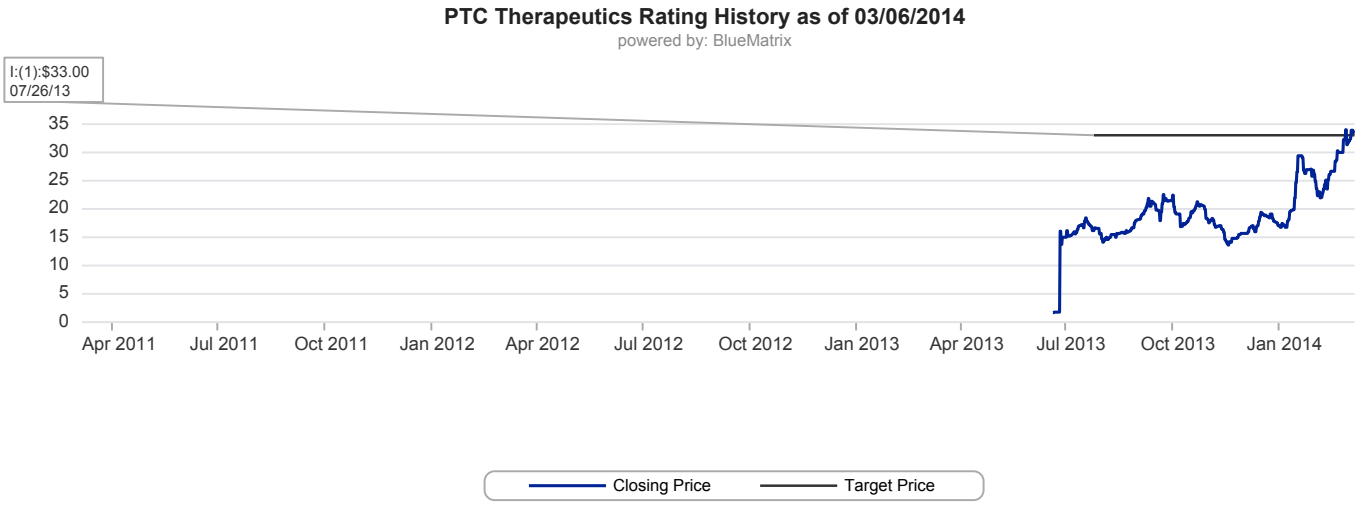
Cowen And Company Rating Definitions

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.



Cempra Rating History as of 03/06/2014
powered by: BlueMatrix

Ocera Therapeutics Inc Rating History as of 03/06/2014
powered by: BlueMatrix

Regado Biosciences Inc Rating History as of 03/06/2014
powered by: BlueMatrix

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

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