COWEN
AND COMPANY

Biotechnology

PTC Therapeutics

Equity Research

Earnings Update

May 8, 2014

Price: $17.60 (05/7/2014)

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):	$529.3
Net Debt (MM):	$0.0
Cash/Share:	$8.19
Dil. Shares Out (MM):	30.1
Enterprise Value (MM):	$282.7
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$5.74
FCF Yield:	NA
Dividend:	NA

FY (Dec)	2013E	2014E	2015E
Earnings Per Share			
Q1	$(2.08)A	$(0.58)A	-
Q2	$(5.51)A	$(0.76)	-
Q3	$(0.19)A	$(0.77)	-
Q4	$(0.75)A	$(0.78)	-
Year	$(5.18)A	$(2.92)	$(2.95)
P/E	NM	NM	NM
Consensus EPS	$(3.78)	$(2.69)	$(2.84)

Consensus source: Thomson Reuters

Revenue (MM)			
Q1	$7.1A	$9.2A	-
Q2	$6.9A	$1.9	-
Q3	$16.3A	$2.1	-
Q4	$4.4A	$2.2	-
Year	$34.7A	$15.4	$11.0
EV/S	8.1x	18.4x	25.7x

Clinical Development Progressing Nicely On All Fronts

The Cowen Insight

PTC reported 1Q14 financials and is making steady progress on all fronts of clinical development. We continue to be bullish on the outcome from the ongoing confirmatory Phase III study of ataluren for nmDMD. With extensive expertise in protein expression modulation and a robust pipeline, PTC has strong long-term growth potential, in our opinion, We reiterate our Outperform rating and $40 PT.

Ataluren Confirmatory Trial in DMD (ACT DMD) is on track

Patient enrollment will be completed by mid-2014 with top-line data expected in mid-2015. The final decision from the CHMP is expected in 2Q14 but PTC maintains its primary focus on completing ACT DMD. We model full approvals for ataluren in both the U.S. and the EU in 2016 based on positive results from the confirmatory study, and treat conditional approval in the EU as upside potential. The confirmatory study was designed based on the understanding of the natural history of DMD that PTC gained from the completed Phase IIb study, so we believe the clinical risk has been reduced to some degree. Ataluren is the only drug candidate in clinical development for nonsense mutations so it will face little competition upon entering the market.

Follow on programs for ataluren and SMA program are progressing well

PTC plans to initiate the Ataluren Confirmatory Trial in nmCF (ACT CF) with a similar design to the previously completed Phase III study. Patients on chronic inhaled tobramycin will be excluded since the antibiotic was found to inhibit ataluren's activity, likely through competing for the same binding site in the ribosomes. A subgroup analysis of the data from the first Phase III study, excluding patients on tobramycin, demonstrated a statistically significant improvement in lung function.Therefore, much like ACT DMD, ACT CF is also designed for an increased chance of success. Additionally, PTC expects to initiate a Phase II proof-of-concept study of ataluren in an additional indication in 2H14. The selection of the indication will be based on factors such as medical need, established clinical endpoints, and available biomarkers for evaluation of clinical improvement. We view ataluren not only as a sole product candidate but also as a pipeline since approximately 11% of monogenic disorders are caused by nonsense mutations and therefore can potentially be addressed by ataluren. Finally, a Phase I study, as well as natural history and biomarker observational studies in SMA patients, are ongoing in a collaboration between PTC, Roche and the SMA Foundation. We believe SMA represents a market with significant potential and that ataluren will be a major player.

A strong balance sheet provides solid support for clinical success

PTC ended 1Q14 with approximately $246.6MM in cash, cash equivalents and marketable securities after completing an equity financing in February 2014. We believe the strengthened balance sheet should sustain the company's operations through 2016. PTC reported 1Q14 EPS of ($0.58) as compared to our estimate of ($0.38) and the consensus of ($0.46).

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
- Initiation of proof-of-concept studies of ataluren for an additional indication in 2H14

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 (05/7/2014)	Price Target	Rating
Acadia Pharmaceuticals	ACAD	$18.51	$33.00	Outperform
Intra-Cellular Therapies	ITCI	$15.42	$28.00	Outperform
Horizon Pharma	HZNP	$13.40	$20.00	Outperform

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

	2011A	2012A	Q1'13A	Q2'13A	Q3'13A	Q4'13A	2013A	Q1'14A	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	9.1	1.8	2.0	2.1	15.0
Grant revenue	6.5	5.2	1.1	1.0	0.8	0.5	3.4	0.1	0.1	0.1	0.1	0.4
Total Revenues and Non-Cash Cancellation Revenue	**105.4**	**33.9**	**7.1**	**6.9**	**16.3**	**4.4**	**34.7**	**9.2**	**1.9**	**2.1**	**2.2**	**$15.4**
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	15.9	16.8	17.5	17.8	68.0
General and Administrative	16.2	14.6	4.5	6.6	6.7	7.5	25.2	7.5	8.0	8.1	8.4	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**	**23.4**	**24.8**	**25.6**	**26.2**	**100.0**
Income (Loss) from Operations	**30.6**	**(26.8)**	**(8.6)**	**(14.5)**	**(4.3)**	**(18.1)**	**(45.4)**	**(14.2)**	**(22.9)**	**(23.5)**	**(24.0)**	**(84.6)**
Other non-operating income (loss)												
Interest income (expense), net	(2.4)	(1.2)	(6.2)	(0.1)	0.0	0.2	(6.1)	0.2	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	0.3	0.4
Income (loss) from operations before tax benefit	**28.6**	**(26.2)**	**(14.7)**	**(14.6)**	**(4.4)**	**(17.9)**	**(51.6)**	**(14.1)**	**(22.8)**	**(23.4)**	**(23.7)**	**(84.2)**
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)**	**(14.1)**	**(22.8)**	**(23.4)**	**(23.7)**	**(84.2)**
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)**	**(14.1)**	**(22.8)**	**(23.4)**	**(23.7)**	**(84.2)**
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)**	**(0.58)**	**(0.76)**	**(0.77)**	**(0.78)**	**($2.92)**
Net Income (Loss) per Share - Diluted	**4.55**	**42.50**	**(1.83)**	**(5.51)**	**(0.19)**	**(0.75)**	**(5.18)**	**(0.58)**	**(0.76)**	**(0.77)**	**(0.78)**	**($2.92)**
Weighted average common shares outstanding - basic	0.001	0.003	14.2	2.6	23.8	23.8	12.8	24.5	30.1	30.3	30.5	28.8
Weighted average common shares outstanding - diluted	0.006	0.017	16.1	2.6	23.8	23.8	12.8	24.5	30.1	30.3	30.5	28.8

Source: Cowen and Company

PTC Therapeutics, Inc. 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	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	3.4	0.4	1.0	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**	**$15.4**	**$11.0**	**$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)**	**(84.6)**	**(93.5)**	**(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)**	**(84.2)**	**(93.0)**	**(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)**	**(84.2)**	**(93.0)**	**(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)**	**(84.2)**	**(93.0)**	**(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)**	**($2.92)**	**($2.95)**	**($2.49)**	**$1.19**	**$4.18**	**$6.67**	**$7.63**	**$8.04**	**$8.19**	**$9.21**	**$9.66**	**$9.24**
Net Income (Loss) per Share - Diluted	**4.55**	**42.50**	**(5.18)**	**($2.92)**	**($2.95)**	**($2.49)**	**$1.14**	**$3.99**	**$6.38**	**$7.31**	**$7.71**	**$7.86**	**$8.85**	**$9.28**	**$8.89**
Weighted average common shares outstanding - basic	0.001	0.003	12.8	28.8	31.5	37.8	38.5	40.0	41.5	43.0	44.0	45.0	46.0	47.0	48.0
Weighted average common shares outstanding - diluted	0.006	0.017	12.8	28.8	31.5	37.8	40.4	41.9	43.4	44.9	45.9	46.9	47.9	48.9	49.9

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
ACAD	Acadia Pharmaceuticals
HZNP	Horizon Pharma
ITCI	Intra-Cellular Therapies
PTCT	PTC Therapeutics

Analyst Certification

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Intra-Cellular Therapies 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 Non-IB services.

PTC Therapeutics, Acadia Pharmaceuticals, Horizon Pharma and Intra-Cellular Therapies 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, Acadia Pharmaceuticals and Horizon Pharma.

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Cowen and Company, LLC and/or its affiliates received in the past 12 months compensation for investment banking services from PTC Therapeutics, Acadia Pharmaceuticals, Horizon Pharma and Intra-Cellular Therapies.

Cowen and Company, LLC and/or its affiliates managed or co-managed a public offering of PTC Therapeutics, Acadia Pharmaceuticals, Horizon Pharma and Intra-Cellular Therapies within the past twelve months.

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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

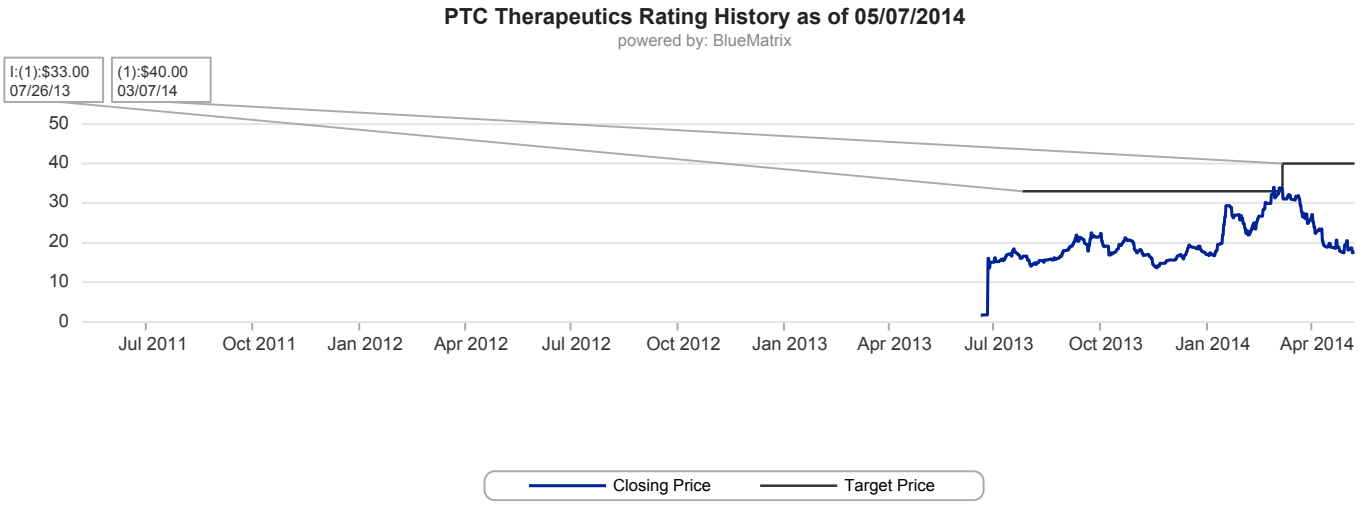
Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	407	57.08%	85	20.88%
Hold (b)	288	40.39%	8	2.78%
Sell (c)	18	2.52%	1	5.56%

(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.



Acadia Pharmaceuticals Rating History as of 05/07/2014

powered by: BlueMatrix

Horizon Pharma Rating History as of 05/07/2014

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Intra-Cellular Therapies Rating History as of 05/07/2014

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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 | S=Suspended

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