COWEN
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Biotechnology

PTC Therapeutics

Equity Research

Company Update

May 23, 2014

Price: $15.32 (05/22/2014)
Price Target: $45.00 (Prior $40.00)

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: PTCT
52-Week Range:	$34.65 - 13.04
Market Cap (MM):	$460.7
Net Debt (MM):	$0.0
Cash/Share:	$8.21
Dil. Shares Out (MM):	30.1
Enterprise Value (MM):	$213.7
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$8.33
FCF Yield:	NA
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	$(2.08)	$(0.58)A	-
Prior Q1	-	-	-
Q2	$(5.51)	$(0.76)	-
Prior Q2	-	-	-
Q3	$(0.19)	$(0.77)	-
Prior Q3	-	-	-
Q4	$(0.75)	$(0.86)	-
Prior Q4	-	*$(0.78)*	-
Year	$(5.18)	$(3.01)	$(2.26)
Prior Year	-	*$(2.92)*	*$(2.95)*
P/E	NM	NM	NM
Consensus EPS	$(3.78)	$(2.89)	$(2.96)
Prior Year	-	*$(2.69)*	*$(2.84)*

Consensus source: Thomson Reuters

Revenue (MM)			
Year	$34.7	$15.4	$38.3
Prior Year	-	-	*$11.0*
EV/S	6.2x	13.9x	5.6x

CHMP Decision A Huge Win For PTC

The Cowen Insight

This morning, PTC announced that the CHMP recommended the conditional approval of Translarna (the brand name of ataluren) for the treatment of nmDMD. We project the drug to be available in 2015 and have adjusted our model accordingly. We reiterate our Outperform rating on PTC shares and raise our 12-month price target from $40 to $45 based on earlier than expected revenue from the EU.

Conditional Approval in the EU supports a strong clinical profile of Translarna

The final decision from the European Commission (EC) is expected within three months and we believe the EC will follow the recommendation of the CHMP to grant the official conditional approval of Translarna, given the devastating nature of DMD, lack of treatment options, and the strong efficacy and safety data from the 174-patient Phase IIb clinical trial. We do not expect the conditional approval to affect the timeline of the ongoing Phase III confirmatory study, which is expected to complete patient enrollment in mid-2014 and report top-line data in mid-2015. And our optimism on the positive outcome from the confirmatory study and the final full approvals in both the U.S. and the EU has been strengthened by the positive opinion from the CHMP. We continue to believe that the Phase III study has been optimally designed, based on accumulating natural history study data in DMD patients, to maximize the clinical benefit of Translarna.

We remain conservative on EU commercialization estimates

PTC has initiated commercial preparations for a potential EU conditional approval even though management had repeatedly cautioned investors about the low likelihood of an actual conditional approval. In our adjusted model, we project PTC to launch Translarna and book full year revenue in 2015. We model an annual cost of $175,000, which we believe is conservative even for the EU market. And we do not believe there will be a significant reimbursement hurdle since there is simply no treatment available for DMD and this disease is associated with high mortality and morbidity. In the U.S., we maintain our previous projections of the U.S. timeline, with Translarna coming onto the market in mid-2016 after a full approval. Our new price target of $45 is the average of values suggested by a discounted earnings model based on projected 2018 EPS, a DCF model, and a clinical NPV model.

We expect PTC to file for EU conditional approval for nmCF in 2H14

We believe the positive opinion from the CHMP on nmDMD should provide PTC with confidence for a conditional approval filing for nmCF. Although we feel the results from the completed Phase III clinical trial are convincing, we model only full approvals in the U.S. and the EU after the planned Phase III confirmatory study. Similar to the nmDMD study, the nmCF confirmatory study will have specific inclusion/exclusion criteria to allow PTC to target the optimal patient population to maximize the chances for success.

Please see addendum of this report for important disclosures.

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, suggests that ataluren can address a combined market of approx. $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 nmCF in the EU

Upside Scenario

- PTC also receives conditional approval for ataluren for 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 (05/22/2014)	Price Target	Rating
Acadia Pharmaceuticals	ACAD	$19.57	$33.00	Outperform
Intra-Cellular Therapies	ITCI	$15.01	$28.00	Outperform
Horizon Pharma	HZNP	$13.40	$20.00	Outperform

PTC Therapeutics, Inc. Revenue Buildup Model

	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Ataluren for Duchenne Muscular Dystrophy (DMD)											
U.S.											
Annual number of births (MM) (-1% annually - economic pressures)		4.0	4.0	3.9	3.9	3.8	3.8	3.8	3.7	3.7	3.7
Incidence of DMD in male infants		1/3,500	1/3,500	1/3,500	1/3,500	1/3,500	1/3,500	1/3,500	1/3,500	1/3,500	1/3,500
Annual incidence of DMD		571	566	560	554	549	543	538	533	527	522
Average life span of DMD patients		25	26	27	28	29	30	31	32	33	34
U.S. prevalence of DMD		14,286	14,709	15,122	15,525	15,918	16,303	16,678	17,043	17,400	17,748
Average age of diagnosis		5	5	5	5	5	5	5	5	5	5
Number of diagnosed DMD patients in the U.S.		11,429	11,880	12,321	12,753	13,174	13,586	13,988	14,380	14,764	15,138
% of DMD patients with nonsense mutations		*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*
Number of U.S. DMD patients amenable to treatment with ataluren		1,486	1,544	1,602	1,658	1,713	1,766	1,818	1,869	1,919	1,968
% market penetration by ataluren		*30.0%*	*40.0%*	*45.0%*	*50.0%*	*55.0%*	*60.0%*	*65.0%*	*70.0%*	*70.0%*	*70.0%*
Number of U.S. DMD patients receiving ataluren treatment		446	618	721	829	942	1,060	1,182	1,309	1,344	1,378
AWP		$125,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000
U.S. Total Ataluren Revenue from DMD ($MM)		**$55.7**	**$154.4**	**$180.2**	**$207.2**	**$235.5**	**$264.9**	**$295.5**	**$327.2**	**$335.9**	**$344.4**
EU											
EU population (MM)	509.0	510.0	511.1	512.2	513.3	514.3	515.4	516.5	517.6	518.7	519.8
EU annual number of birth per 1,000 population	10.3	10.3	10.2	10.1	10.0	9.9	9.8	9.7	9.6	9.5	9.4
EU annual number of birth (MM)	5.2	5.3	5.2	5.2	5.1	5.1	5.0	5.0	5.0	4.9	4.9
Annual incidence of DMD	749	750	744	739	733	727	721	716	710	704	699
EU prevalence of DMD	18,724	18,761	19,357	19,943	20,518	21,083	21,637	22,182	22,717	23,241	23,757
Number of dianosed DMD patients in EU	14,979	15,009	15,634	16,250	16,854	17,448	18,031	18,604	19,167	19,720	20,263
% of DMD patients with nonsense mutations	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*	*13.0%*
Number of EU DMD patients eligible for ataluren treatment	1,947	1,951	2,032	2,112	2,191	2,268	2,344	2,419	2,492	2,564	2,634
% market penetration by ataluren	*8.0%*	*15.0%*	*20.0%*	*25.0%*	*30.0%*	*35.0%*	*40.0%*	*45.0%*	*50.0%*	*55.0%*	*55.0%*
Number of EU DMD patients receiving ataluren treatment	156	293	406	528	657	794	938	1,088	1,246	1,410	1,449
AWP	175000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000
EU Total Ataluren Revenue ($MM)	**$27.3**	**$51.2**	**$71.1**	**$92.4**	**$115.0**	**$138.9**	**$164.1**	**$190.5**	**$218.0**	**$246.7**	**$253.5**
WW Total Ataluren DMD Revenue ($MM)	**$27.3**	**$106.9**	**$225.6**	**$272.6**	**$322.3**	**$374.4**	**$429.0**	**$486.0**	**$545.2**	**$582.6**	**$597.9**
Ataluren for Cystic Fibrosis (CF)											
U.S.											
U.S. prevalence of CF			33,295	33,794	34,301	34,816	35,338	35,868	36,406	36,952	37,507
% of CF patients with nonsense mutations			*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*
Number of U.S. nmCF patients			3,330	3,379	3,430	3,482	3,534	3,587	3,641	3,695	3,751
% of nmCF patients who can discontinue TOBI			*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*
Number of U.S. CF patients amenable to treatment with ataluren			1,665	1,690	1,715	1,741	1,767	1,793	1,820	1,848	1,875
% market penetration by ataluren			*5.0%*	*15.0%*	*25.0%*	*30.0%*	*35.0%*	*40.0%*	*45.0%*	*50.0%*	*50.0%*
Number of U.S. CF patients receiving ataluren treatment			83	253	429	522	618	717	819	924	938
AWP			$125,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000
U.S. Total Ataluren Revenue from CF ($MM)			**$10.4**	**$63.4**	**$107.2**	**$130.6**	**$154.6**	**$179.3**	**$204.8**	**$231.0**	**$234.4**
EU											
EU prevalence of CF			44,175	44,617	45,063	45,514	45,969	46,428	46,893	47,362	47,835
% of CF patients with nonsense mutations			*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*
Number of U.S. nmCF patients			4,418	4,462	4,506	4,551	4,597	4,643	4,689	4,736	4,784
% of nmCF patients who can discontinue TOBI			*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*	*50.0%*
Number of EU CF patients amenable to treatment with ataluren			2,209	2,231	2,253	2,276	2,298	2,321	2,345	2,368	2,392
% market penetration by ataluren			*2.5%*	*10.0%*	*20.0%*	*25.0%*	*30.0%*	*35.0%*	*40.0%*	*40.0%*	*40.0%*
Number of U.S. CF patients receiving ataluren treatment			55	223	451	569	690	812	938	947	957
AWP			$87,500	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000	$175,000
EU Total Ataluren Revenue from CF ($MM)			**$4.8**	**$39.0**	**$78.9**	**$99.6**	**$120.7**	**$142.2**	**$164.1**	**$165.8**	**$167.4**
WW Total Ataluren CF Revenue ($MM)			**$15.2**	**$102.4**	**$186.1**	**$230.1**	**$275.3**	**$321.5**	**$368.9**	**$396.7**	**$401.8**
WW Total Ataluren Sales Revenues to PTC ($MM)	**$27.3**	**$106.9**	**$240.8**	**$375.0**	**$508.3**	**$604.5**	**$704.3**	**$807.5**	**$914.1**	**$979.3**	**$999.8**

Source: Cowen and Company

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	2.5	2.5
Total Operating Expenses	**74.8**	**60.8**	**15.7**	**21.3**	**20.6**	**22.5**	**80.1**	**23.4**	**24.8**	**25.6**	**28.7**	**102.5**
Income (Loss) from Operations	**30.6**	**(26.8)**	**(8.6)**	**(14.5)**	**(4.3)**	**(18.1)**	**(45.4)**	**(14.2)**	**(22.9)**	**(23.5)**	**(26.5)**	**(87.1)**
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)**	**(26.2)**	**(86.7)**
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)**	**(26.2)**	**(86.7)**
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)**	**(26.2)**	**(86.7)**
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)**	**(0.86)**	**($3.01)**
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)**	**(0.86)**	**($3.01)**
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	27.3	106.9	240.8	375.0	508.3	604.5	704.3	807.5	914.1	979.3	999.8
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**	**$38.3**	**$106.9**	**$240.8**	**$375.0**	**$508.3**	**$604.5**	**$704.3**	**$807.5**	**$914.1**	**$979.3**	**$999.8**
Operating Expenses															
COGS	0.0	0.0	0.0	0.0	0.0	16.0	33.7	48.8	61.0	66.5	70.4	80.7	91.4	97.9	100.0
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	2.5	8.0	25.0	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**	**102.5**	**110.0**	**146.0**	**160.7**	**176.3**	**194.2**	**204.8**	**220.0**	**241.0**	**262.7**	**281.7**	**295.6**
Income (Loss) from Operations	**30.6**	**(26.8)**	**(45.4)**	**(87.1)**	**(71.7)**	**(39.1)**	**80.1**	**198.8**	**314.1**	**399.8**	**484.2**	**566.5**	**651.4**	**697.7**	**704.2**
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)**	**(86.7)**	**(71.2)**	**(38.6)**	**80.6**	**199.3**	**314.6**	**400.3**	**484.7**	**567.0**	**651.9**	**698.2**	**704.7**
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)**	**(86.7)**	**(71.2)**	**(38.6)**	**80.6**	**199.3**	**314.6**	**400.3**	**484.7**	**567.0**	**651.9**	**698.2**	**704.7**
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)**	**(86.7)**	**(71.2)**	**(38.6)**	**80.6**	**199.3**	**314.6**	**400.3**	**484.7**	**567.0**	**651.9**	**698.2**	**704.7**
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	2.4	15.9	37.7	72.0	130.9	198.4	228.2	244.4	246.6
Net Income (Loss) per Share - Basic	**23.95**	**219.76**	**($5.18)**	**($3.01)**	**($2.26)**	**($1.02)**	**$2.03**	**$4.58**	**$6.67**	**$7.63**	**$8.04**	**$8.19**	**$9.21**	**$9.66**	**$9.54**
Net Income (Loss) per Share - Diluted	**4.55**	**42.50**	**($5.18)**	**($3.01)**	**($2.26)**	**($1.02)**	**$1.94**	**$4.38**	**$6.38**	**$7.31**	**$7.71**	**$7.86**	**$8.85**	**$9.28**	**$9.18**
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

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.

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PTC Therapeutics, Acadia Pharmaceuticals, Horizon Pharma and Intra-Cellular Therapies have been client(s) of Cowen and Company, LLC in the past 12 months.

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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

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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

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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.

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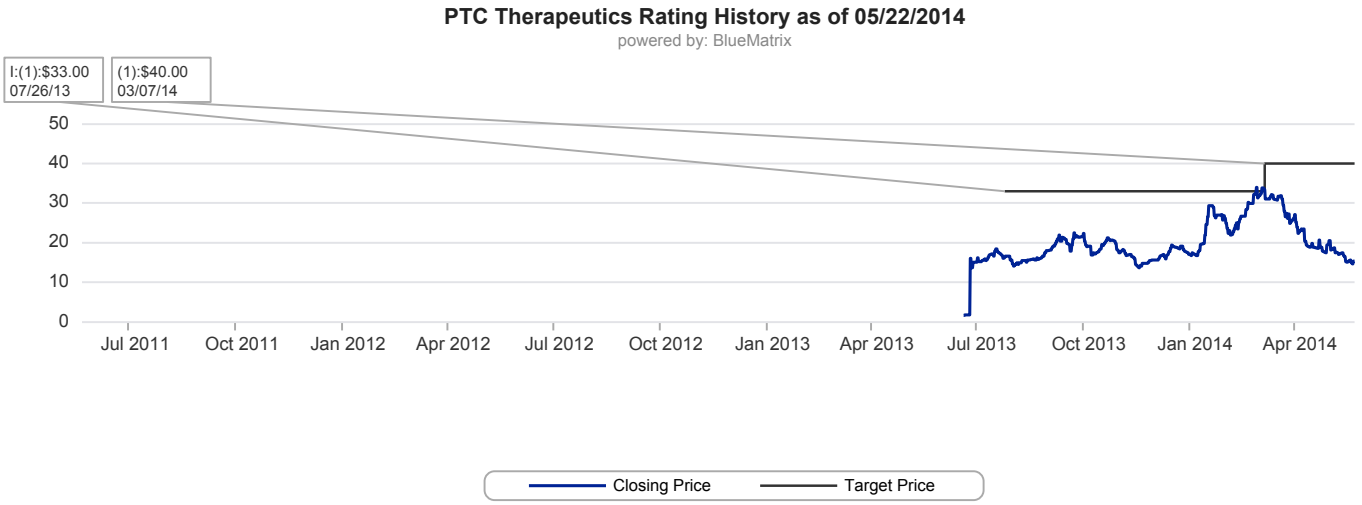

Acadia Pharmaceuticals Rating History as of 05/22/2014

powered by: BlueMatrix

Horizon Pharma Rating History as of 05/22/2014

powered by: BlueMatrix

Intra-Cellular Therapies Rating History as of 05/22/2014

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 | S=Suspended

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