

FLASH NOTE

Biotechnology

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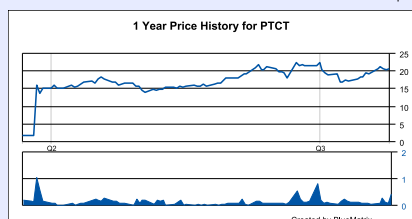
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Recommendation

Rating:	Outperform
Price Target:	\$33.00

Stock Statistics as of 10/25/2013

Price:	\$20.71
52W Range:	\$24.38-\$13.04
Shares Out (MM):	24.9
Market Cap (MM):	\$516.1
Net Debt (MM):	\$2.2
Net Cash Per Share:	\$6.65



PTC THERAPEUTICS, INC. (NASDAQ:PTCT)

GEMS In The Pipeline Shine At Analyst Day

Last Friday, PTC hosted an analyst day and the discussion focused squarely on the company's technology platform, namely GEMS (Gene Expression Modulation by Small Molecules), and the company's diversified follow-on programs. Management provided a comprehensive overview of the science behind the versatility of GEMS in targeting a wide variety of Orphan indications such as spinal muscular atrophy (SMA), Huntington's disease, and exon-skipping for Duchenne Muscular Dystrophy (DMD). Management presented encouraging early stage data from these programs as well as from the anti-tumor and anti-infective disease programs. We left the analyst day feeling convinced that PTC, with extensive expertise and a well-established technology platform for identification of small molecule compounds in modulating post-transcriptional control, represents an attractive investment opportunity. Based on the multiple near-term catalysts, we remain bullish on PTC shares and reiterate our Outperform rating.

Guidance on the nmDMD timeline is unchanged

Management reiterated that patient enrollment in the ongoing Phase III clinical trial for nmDMD will be complete by mid-2014 and topline data will be available in mid-2015. Management continues to expect the final EMA decision on the conditional approval for ataluren in nmDMD by year-end 2013. We are optimistic that the ongoing Phase III clinical trial will be successful and will be sufficient to support a full approval in both the U.S. and EU. Therefore, our model does not include an EU conditional approval and we treat the possibility as upside potential for the company. No specific updates regarding the nmCF program.

Roche is taking an active lead in the SMA program

PTC announced the selection of a development candidate in August 2013 and toxicology studies and chemical processing are currently underway. With a development team and a clinical team already in place, PTC and Roche are planning a rapid proof-of-concept study "as soon as possible". At the recent 16th annual Cowen Therapeutics Conference, surveyed physicians and the audience viewed PTC's SMN2 splicing modifier as the second most promising candidate for SMA, just following ISIS/Biogen's compound, which is already in Phase II clinical development. PTC's small molecules have demonstrated good oral bioavailability, dose dependent increases in SMN protein levels, efficient penetration of blood brain barrier, and phenotypic rescue in SMA model mice. We believe PTC will be one of the major players in the SMA space over time.

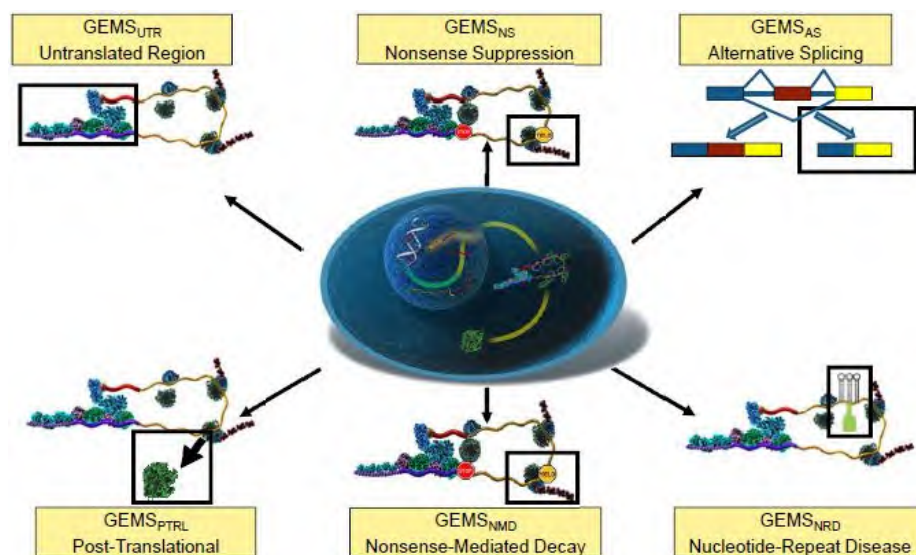
Alaluren, both a product and a pipeline, will remain the near-term focus

Roughly 11% of monogenic disorders are caused by nonsense mutations and can potentially be addressed by ataluren. PTC is actively engaged in collaborations to evaluate ataluren in animal models of multiple Orphan disorders. Based on factors such as medical need and established clinical endpoints, PTC believes the next indication for ataluren may be chosen in 2014. We believe the strong safety profile that ataluren has demonstrated and the large patient database to date will help expedite future clinical development for additional indications.

Please see addendum of this report for important disclosures.



Exhibit 1. GEMS Technology's Strong Versatility



Source: PTC Therapeutics, Inc.

Anti-tumor and antibacterial programs further diversify PTC's pipeline

PTC is developing PTC596, an oral compound that specifically targets tumor stem cells, and a novel class of bacterial synthesis inhibitors with grant funding from the Wellcome Trust Foundation. PTC presented highly intriguing preclinical data from both programs at the analyst day. PTC596 demonstrated strong *ex vivo* and *in vivo* reductions of tumor stem cells but had no impact on normal stem cells. IND-enabling studies are currently ongoing and glioblastoma will be the lead indication for the program. The novel bacterial synthesis inhibitor class includes both compounds with high specificity and broad spectrum compounds that are active against gram negative strains or MRSA. PTC plans to choose gonorrhea as the first indication based on the simplicity of the clinical trial design and finalization of the development candidate is expected in 1H14.

We believe PTC will continue to focus on Orphan indications with the versatile GEMS technology platform and that ataluren will remain the key value driver for the company. However, the anti-tumor and anti-infective disease programs, both addressing large markets and good candidates for partnerships, provide PTC with additional upside potential.

**Exhibit 2. 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	2H15/2016
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
		Top-line data from the confirmatory Phase III clinical trial	2H15/2016

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

STOCKS MENTIONED IN IMPORTANT DISCLOSURES

Ticker	Company Name
PTCT	PTC Therapeutics, Inc.

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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

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

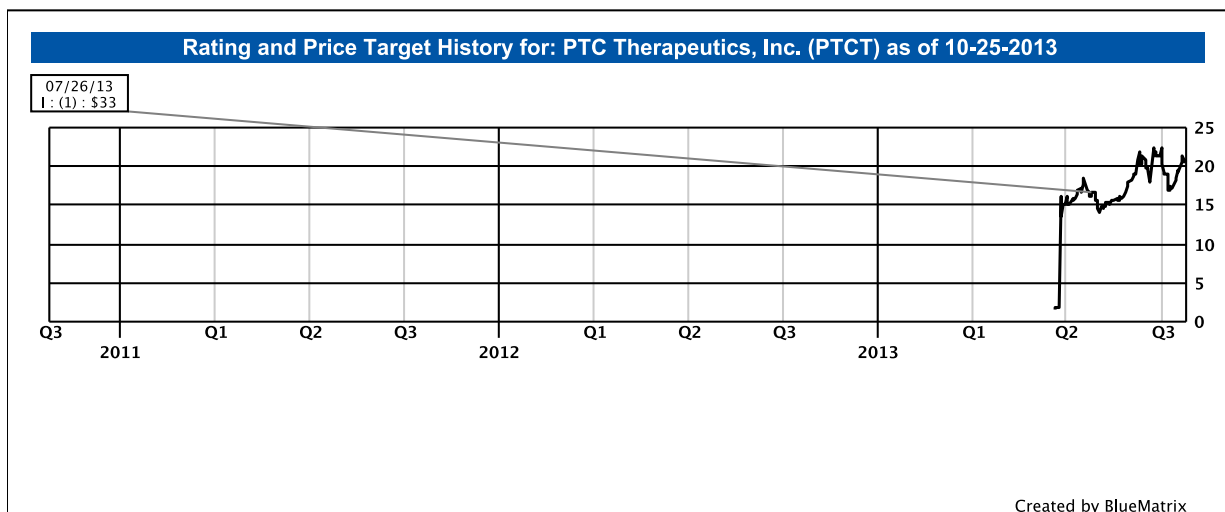
COWEN AND COMPANY RATING ALLOCATION

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	394	58.72%	54	13.71%
Hold (b)	255	38.00%	5	1.96%
Sell (c)	22	3.28%	1	4.55%

(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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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