

## FLASH NOTE

Biotechnology

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### Recommendation

Rating: Outperform  
Price Target: \$45.00

### Stock Statistics as of 11/22/2013

Price: \$23.89  
52W Range: \$30.95-\$14.50  
Shares Out (MM): 39.2  
Market Cap (MM): \$948.7  
Net Debt (MM): \$(131.3)



## PORTOLA PHARMACEUTICALS INC

(NASDAQ:PTLA)

# Andexanet Receives Breakthrough Therapy Designation

This morning Portola announced that it has been granted breakthrough therapy designation for andexanet alpha, its Factor Xa inhibitor antidote. According to the FDA, breakthrough therapy designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. In order to be granted breakthrough therapy designation a candidate must have demonstrated preliminary clinical evidence of improvement on at least one clinically significant endpoint over available therapy. Breakthrough therapy is intended to make as efficient as possible the development and regulatory review of the candidate, and senior FDA management are to provide guidance on the development program of the candidate. By our count, there have been at least 33 products designated as breakthrough therapies since the program began in 2012.

Portola will report additional data from the Phase II trials of andexanet alfa and rivaroxaban, and andexanet and enoxaparin, over the next two quarters, including a poster presentation of data from the rivaroxaban trial at ASH in December. Portola will finalize the design of andexanet's pivotal trial after analyzing the full Phase II results, but continues to expect to seek approval for andexanet using PK/PD endpoints in Phase III under an Accelerated Approval pathway. A subsequent Phase IV study will study andexanet in patients with uncontrolled bleeding, or in need of emergency surgery. Both the Phase III and IV trials are on track to begin during 2014.

We think this morning's designation is significant for three reasons. First, it highlights the fact that andexanet addresses an important unmet medical need, and that the FDA sees some urgency to speed its development. Second, it suggests the data generated thus far in Phase II are reasonably likely to predict a clinical benefit. Last, the designation will help ensure that andexanet's pivotal trial program is developed with the input and agreement of the FDA, and therefore there should be little doubt that the trial design and endpoints will be sufficient for FDA approval.

We continue to think Portola is undervalued for the potential of andexanet and betrixaban, and consider it a top small cap pick.

Please see addendum of this report for important disclosures.



## 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

Much of Portola's valuation rests on the potential of two candidates, Betrixaban and PRT4445. Projecting future sales for any product is difficult, and this is particularly the case for candidates that are still in clinical development. Portola's stock could be impacted by changes in the regulatory, commercial, or competitive environment for either. In particular, Betrixaban is in development in an indication for which candidates of the same mechanism have failed clinical trials; Betrixaban's clinical development must therefore be considered risky. The studies necessary for PRT4445 to receive regulatory approval have yet to be defined; there is therefore risk that its clinical development takes longer than we anticipate.



## Addendum

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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

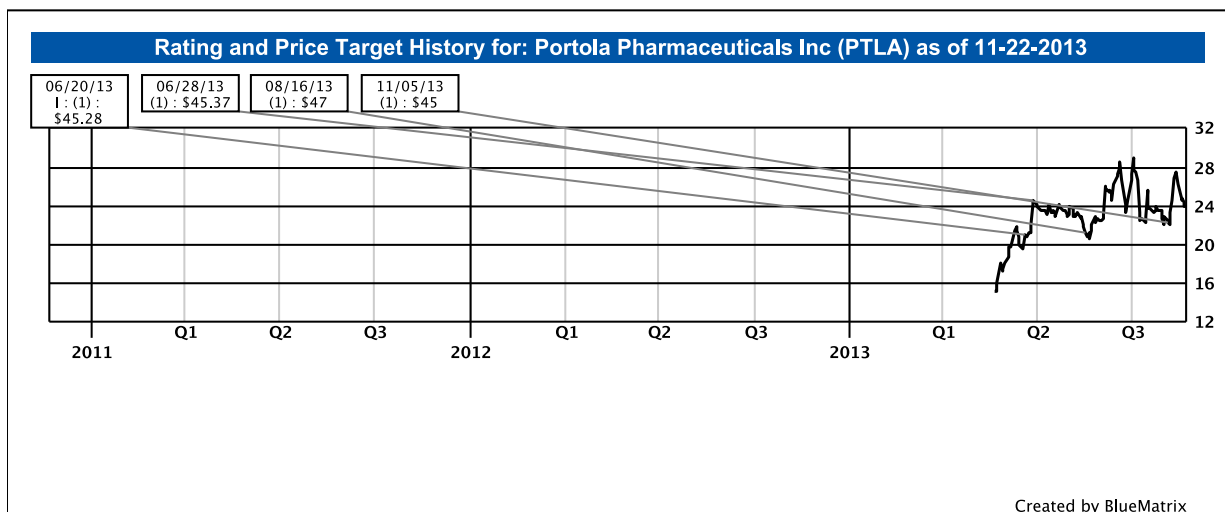
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##### ***Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13***

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