

Equity Research

December 9, 2013

**Price: \$24.18** (12/9/2013)

**Price Target: \$45.00**

**OUTPERFORM (1)**

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Company Quick Take

## *Andexanet Continues To Look Promising In New ASH Data*

### **The Cowen Insight**

Portola presented new data from a Ph. II trial of andexanet alfa at ASH in which it produced dose-dependent decreases in both anti-Factor Xa activity and unbound Xarelto. We continue to think that andexanet represents a very promising \$500MM+ opportunity, and that Portola is undervalued based on the promise of andexanet and betrixaban. We consider PTLA a top small cap pick.

### **Additional Data From Andexanet's Phase II With Xarelto Presented At ASH.**

The double-blind, placebo-controlled Phase II treated 36 healthy volunteers with 20mg QD Xarelto for six days, and then randomized them 6:3 to receive andexanet alfa in 4 dosing cohorts. The first three cohorts received a single IV bolus of andexanet alfa at 210mg, 420mg, or 600mg, while a fourth cohort received a single IV bolus of andexanet alfa at 720mg followed by a 4mg/min infusion for one hour. Data from the 210mg and 420mg cohorts was released in November, and showed reductions of anti-Factor Xa activity of 20% and 53% after 2 minutes. Results from higher dose cohorts and other endpoints were released today at the annual meeting of the American Society for Hematology (ASH). Following the completion of the 600mg and 720mg bolus of andexanet alfa, anti-Factor Xa activity decreased by 70% and 81%, respectively. For all dose groups anti-Factor Xa activity returned to placebo levels approx. two hours after treatment. Plasma concentrations of unbound Xarelto were decreased by 32%, 51%, 75%, and 70%, respectively, relative to pre-andexanet levels. Xarelto-induced inhibition of thrombin generation, prolongation of prothrombin, and activated clotting time approached normal levels in a dose-dependent manner. We find these efficacy data compelling. Andexanet continued to look well tolerated, with no thrombotic events, serious adverse events, or antibodies for Factor X or Xa.

### **Andexanet's Pivotal Trial On Track To Begin During H1:14.**

Portola discussed andexanet's registration program at this afternoon's analyst event. Portola expects to soon meet with the FDA to finalize the design of both the pivotal trial, and Phase IV confirmatory studies. Portola expects the pivotal trial will have as its co-primary endpoints anti-Factor Xa levels and plasma concentrations of unbound Factor Xa inhibitor. A key secondary endpoint will be the restoration of thrombin generation. As andexanet has convincingly shown that it can hit these endpoints in Phase II, we think the pivotal trial is very likely to succeed. Portola is working to design the Phase IV study, and will propose to the FDA either a trial in patients having a spontaneous major bleed, or one in patients in need of emergency surgery. Portola continues to expect to initiate the pivotal trial during H1:14, and to be in a position to launch andexanet during 2016. During today's analyst meeting Portola offered its market analysis suggesting that by 2020 the number of patients presenting to a hospital who could benefit from an antidote in the U.S., EU5 and Japan may approach 500K. Our model assumes that in 2020 50K people will receive andexanet worldwide, resulting in \$355MM in revenue to Portola.

## *Valuation Methodology And Risks*

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### **Valuation Methodology**

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#### **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**

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#### **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**

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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

## Stocks Mentioned In Important Disclosures

Ticker	Company Name
PTLA	Portola Pharmaceuticals Inc

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**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

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**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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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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### Portola Pharmaceuticals Inc Rating History as of 12/06/2013

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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

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