

# Portola Pharmaceuticals, Inc.

## First Quarter 2014 Earnings Results Highlighted by Clinical Progress and Strong Balance Sheet

After the markets closed on Monday, May 12, Portola reported a first-quarter loss of \$0.75 per share, in line with our estimate of a loss of \$0.78 per share and the Street consensus for a loss of \$0.72 per share. We discuss the highlights from the earnings call as well as our thoughts below. An analysis showing the variance between our estimates and the company's actual results are summarized in exhibit 1.

### Key Highlights Following Management Call With Investors

- **Management announced a change to its commercial-scale manufacturing strategy for andexanet alfa, but timelines remain unchanged.** Due to lower-than-expected yields with the commercial-scale manufacturing for andexanet alfa, management has decided to alter its strategy to maintain its timelines for the biologics license application (BLA) submission expected in the second half of 2015. The new strategy involves maintaining the supplier that supplied the clinical material for the trials through the regulatory application process and into the early stages of the commercial launch, and then filing a supplemental BLA to transition to a new supplier with greater scale and a lower cost of sales. While we acknowledge there is always risk in modifying commercial-scale plans while in late-stage clinical trials and ahead of a regulatory submission, we point out that the processes between the suppliers' facilities are the same.
- **Management reiterated 2014 financial guidance.** Operating expenses continue to be between \$145 million and \$160 million in 2014. While we acknowledge this is a meaningful increase versus 2013, we believe the company's investment in its three most advanced clinical compounds is warranted and has the potential to create significant shareholder value over the long term. Management also reiterated that it expects to end the year with \$185 million-\$200 million in cash. At the end of the last quarter, Portola had \$306 million in cash, which it expects to take it into the second quarter of 2016. We believe Portola has an active news calendar during the next 12-24 months and note that its cash is sufficient to take it through important late-stage clinical catalysts, particularly with betrixaban and andexanet alfa.
- **We expect numerous catalysts over the next 12-24 months.** We anticipate meaningful updates with Portola's three most advanced, wholly owned candidates in clinical development over the next two years. For betrixaban, we highlight the data and safety review in 2014, the trial's enrollment completion in 2015, and results in early 2016. For andexanet alfa, we note that results from Phase III trials in combination with Eliquis and Xarelto will be available later in the year and in early 2015, followed by a potential regulatory submission if the data is positive. For cerdulatinib, we expect Phase I/II data throughout 2014. We believe the number of upcoming catalysts is illustrative of the progress Portola is making in advancing the compounds, and we believe positive news with each of these programs has the potential to drive upside for the company.

We maintain our Outperform rating on Portola shares.

*Portola Pharmaceuticals is a late-stage, small-cap therapeutics company based in South San Francisco, California. It focuses on developing drugs for the treatment of various thrombotic and hematologic disorders.*

John Sonnier  
+1 312 364 8224  
jsonnier@williamblair.com

Richard M. Reznick, Ph.D.  
+1 312 364 8054  
rreznick@williamblair.com

Please consult pages 4-5 of this report for all disclosures. Analyst certification is on page 4. William Blair & Company, L.L.C. does and seeks to do business with companies covered in its research reports. As a result, investors should be aware that the firm may have a conflict of interest that could affect the objectivity of this report. Investors should consider this report as a single factor in making an investment decision.

May 12, 2014

Stock Rating: **Outperform**  
Company Profile: **Aggressive Growth**

Symbol: PTLA (NASDAQ)  
Price: \$23.95 (52-Wk.: \$15-\$31)  
Market Value (mil.): \$978  
Fiscal Year End: December  
Long-Term EPS Growth Rate:  
Dividend/Yield: None

	2013A	2014E	2015E
<b>Estimates</b>			
EPS FY	\$-3.35	\$-3.39	\$-3.56
CY		\$-3.39	\$-3.56
Sales (mil.)	11	6	5
<b>Valuation</b>			
FY P/E	NM	NM	NM
CY P/E		NM	NM

<b>Trading Data (FactSet)</b>	
Shares Outstanding (mil.)	39
Float (mil.)	31
Average Daily Volume	314,092

<b>Financial Data (FactSet)</b>	
Long-Term Debt/Total Capital (MRQ)	0.0
Book Value Per Share (MRQ)	7.2
Return on Equity (TTM)	-32.4

### Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

## Portfolio Manager Summary

After the markets closed today, Portola reported a first-quarter loss of \$0.75 per share, in line with our estimate for a loss of \$0.78 per share and the Street consensus for a loss of \$0.72 per share. We discuss the highlights from the earnings call as well as our thoughts below. An analysis of the variance between our estimates and the company's actual results for the quarter are summarized in exhibit 1.

### Exhibit 1 Portola Pharmaceuticals, Inc. First Quarter 2014 Variance Analysis (dollars in millions except EPS)

	PTLA Q1 2014A	WB Q1 2014E
Total Revenues	\$2	\$1
R&D	\$28	\$29
SG&A	\$5	\$4
Net Income	(\$31)	(\$32)
EPS	(\$0.75)	(\$0.78)

Sources: Portola reports and William Blair & Company, L.L.C. estimates.

Financially, Portola reiterated 2014 expense guidance of \$145 million-\$160 million. We note this is a meaningful increase versus 2013, but we believe it is warranted given the value in the three most advanced clinical assets. Management also said it expects to end the year with \$185 million to \$200 million in cash. At the end of the last quarter, Portola had \$306 million in cash, which it expects to take it into the second quarter of 2016. We believe this is a sufficient amount of cash to take Portola through important catalysts over the next 12-24 months, which we believe have the potential to create significant shareholder value. We modified our other financial estimates to come more in line with operating trends and management's financial guidance, all of which we detail in exhibit 2.

### Exhibit 2 Portola Pharmaceuticals, Inc. Guidance and Estimates (dollars in millions except EPS)

	WB Previous 2014E	WB Revised 2014E	WB Previous 2015E	WB Revised 2015E	WB Previous 2016E	WB Revised 2016E
Total Revenues	\$3	\$6	\$3	\$5	\$5	\$6
R&D	\$124	\$123	\$134	\$134	\$144	\$144
SG&A	\$22	\$24	\$28	\$28	\$38	\$38
Net Income	(\$142)	(\$140)	(\$158)	(\$156)	(\$177)	(\$176)
EPS	(\$3.44)	(\$3.39)	(\$3.60)	(\$3.56)	(\$3.81)	(\$3.79)

Sources: Portola reports and William Blair & Company, L.L.C. estimates.

We expect continued focus on the company's pipeline and upcoming clinical catalysts. We highlight that in addition to the company's flagship program with betrixaban, there is growing investor interest regarding andexanet alfa. And we note that given existing clinical and regulatory timelines, andexanet alfa might be Portola's first compound eligible for regulatory approval. A timeline illustration of expected upcoming events is presented in exhibit 3.

**Exhibit 3**  
**Portola Pharmaceuticals, Inc.**  
**Timeline**

Date	Drug	Event
2014	Andexanet Alfa	Phase III trial results in combination with Eliquis and Xarelto in uncontrolled bleeding (Q4).
	Andexanet Alfa	Phase IV trial initiation in uncontrolled bleeding (2H).
	Andexanet Alfa	Phase II trial additional results with other Factor Xa inhibitors (Lovenox) in uncontrolled bleeding (June).
	Andexanet Alfa	Phase II trial additional results with other Factor Xa inhibitors (Eliquis) in uncontrolled bleeding (August).
	Andexanet Alfa	Commercial-scale manufacturing progress.
	Betrixaban	Phase III APEX trial third planned Data Safety Monitoring Committee review in venous thromboembolism prevention in acute medical illness.
	Cerdulatinib	Phase I/II trial results in hematologic cancers, including chronic lymphocytic leukemia and non-Hodgkin lymphoma.
2015	PRT2607	Phase II trial initiation in allergic asthma.
	Andexanet Alfa	Phase III trial additional results in combination with Eliquis and Xarelto in uncontrolled bleeding (1H).
	Andexanet Alfa	Regulatory submission in uncontrolled bleeding (2H).
	Andexanet Alfa	Phase II trial additional results with other Factor Xa inhibitors in uncontrolled bleeding.
	Betrixaban	Phase III APEX trial futility analysis in venous thromboembolism prevention in acute medical illness.
2016	Betrixaban	Phase III APEX trial enrollment completion in venous thromboembolism prevention in acute medical illness.
	Betrixaban	Phase III APEX trial results in venous thromboembolism prevention in acute medical illness.
	Betrixaban	Potential regulatory submission for venous thromboembolism prevention in acute medical illness.

Sources: Portola reports.

### Valuation

Portola is trading at \$23.95 with a market cap of \$978 million. We believe that Portola's three advanced, wholly owned clinical compounds offer the potential for significant shareholder value creation due to the robust efficacy and safety observed to date and the significant commercial opportunity in markets with a high unmet medical need.

### Risks

We believe the most important risks for Portola are clinical, regulatory, and financial. As with all biotechnology companies engaged in clinical development, the risk that trials fail is significant, and Portola is engaging in late-stage trials with its two most advanced assets. We believe failures with either of these compounds will weigh on the company's shares. In addition, Portola has no prior experience bringing a compound to market, so we acknowledge that there is regulatory risk as the company navigates that process for the first time. Lastly, Portola continues to spend more money than it generates, so there is the risk that the company will need to access the financial markets again to fund its operations for the future.

### **IMPORTANT DISCLOSURES**

William Blair was a manager or co-manager of a public offering of equity securities for Portola Pharmaceuticals, Inc. within the prior 12 months.

William Blair is a market maker in the security of Portola Pharmaceuticals, Inc. and may have a long or short position.

William Blair intends to seek investment banking compensation in the next three months from Portola Pharmaceuticals, Inc.

Within the past 12 months William Blair has provided or is providing investment banking services to or has an investment services relationship with Portola Pharmaceuticals, Inc.

Additional information is available upon request.

This report is available in electronic form to registered users via R\*Docs™ at [www.rdocs.com](http://www.rdocs.com) or [www.williamblair.com](http://www.williamblair.com).

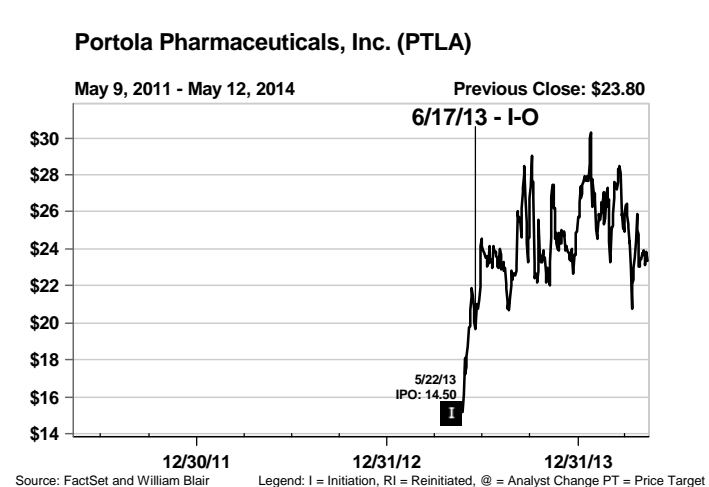
Please contact us at +1 800 621 0687 or consult [williamblair.com/Research-and-Insights/Equity-Research/Coverage.aspx](http://williamblair.com/Research-and-Insights/Equity-Research/Coverage.aspx) for all disclosures.

John Sonnier attests that 1) all of the views expressed in this research report accurately reflect his/her personal views about any and all of the securities and companies covered by this report, and 2) no part of his/her compensation was, is, or will be related, directly or indirectly, to the specific recommendations or views expressed by him/her in this report. We seek to update our research as appropriate, but various regulations may prohibit us from doing so. Other than certain periodical industry reports, the majority of reports are published at irregular intervals as deemed appropriate by the analyst.

DOW JONES: 16,583.34

S&P 500: 1,878.48

NASDAQ: 4,071.87



### **Current Rating Distribution (as of 04/30/14)**

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	66	Outperform (Buy)	14
Market Perform (Hold)	31	Market Perform (Hold)	2
Underperform (Sell)	1	Underperform (Sell)	0

\*Percentage of companies in each rating category that are investment banking clients, defined as companies for which William Blair has received compensation for investment banking services within the past 12 months.

The compensation of the research analyst is based on a variety of factors, including performance of his or her stock recommendations; contributions to all of the firm's departments, including asset management, corporate finance, institutional sales, and retail brokerage; firm profitability; and competitive factors.

### **OTHER IMPORTANT DISCLOSURES**

Stock ratings, price targets, and valuation methodologies: William Blair & Company, L.L.C. uses a three-point system to rate stocks. Individual ratings and price targets (where used) reflect the expected performance of the stock relative to the broader market (generally the S&P 500, unless otherwise indicated) over the next 12 months. The assessment of expected performance is a function of near-, intermediate-, and long-term

company fundamentals, industry outlook, confidence in earnings estimates, valuation (and our valuation methodology), and other factors. Outperform (O) – stock expected to outperform the broader market over the next 12 months; Market Perform (M) – stock expected to perform approximately in line with the broader market over the next 12 months; Underperform (U) – stock expected to underperform the broader market over the next 12 months; not rated (NR) – the stock is not currently rated. The valuation methodologies used to determine price targets (where used) include (but are not limited to) price-to-earnings multiple (P/E), relative P/E (compared with the relevant market), P/E-to-growth-rate (PEG) ratio, market capitalization/revenue multiple, enterprise value/EBITDA ratio, discounted cash flow, and others.

Company Profile: The William Blair research philosophy is focused on quality growth companies. Growth companies by their nature tend to be more volatile than the overall stock market. Company profile is a fundamental assessment, over a longer-term horizon, of the business risk of the company relative to the broader William Blair universe. Factors assessed include: 1) durability and strength of franchise (management strength and track record, market leadership, distinctive capabilities); 2) financial profile (earnings growth rate/consistency, cash flow generation, return on investment, balance sheet, accounting); 3) other factors such as sector or industry conditions, economic environment, confidence in long-term growth prospects, etc. Established Growth (E) – Fundamental risk is lower relative to the broader William Blair universe; Core Growth (C) – Fundamental risk is approximately in line with the broader William Blair universe; Aggressive Growth (A) – Fundamental risk is higher relative to the broader William Blair universe.

The ratings, price targets (where used), valuation methodologies, and company profile assessments reflect the opinion of the individual analyst and are subject to change at any time.

Our salespeople, traders, and other professionals may provide oral or written market commentary or trading strategies—to our clients and our trading desks—that are contrary to opinions expressed in this research. Certain outstanding reports may contain discussions or investment opinions relating to securities, financial instruments and/or issuers that are no longer current. Always refer to the most recent report on a company or issuer before making an investment decision. Our asset management and trading desks may make investment decisions that are inconsistent with recommendations or views expressed in this report. We will from time to time have long or short positions in, act as principal in, and buy or sell the securities referred to in this report. Our research is disseminated primarily electronically, and in some instances in printed form. Electronic research is simultaneously available to all clients. This research is for our clients only. No part of this material may be copied or duplicated in any form by any means or redistributed without the prior written consent of William Blair & Company, L.L.C.

THIS IS NOT IN ANY SENSE A SOLICITATION OR OFFER OF THE PURCHASE OR SALE OF SECURITIES. THE FACTUAL STATEMENTS HEREIN HAVE BEEN TAKEN FROM SOURCES WE BELIEVE TO BE RELIABLE, BUT SUCH STATEMENTS ARE MADE WITHOUT ANY REPRESENTATION AS TO ACCURACY OR COMPLETENESS OR OTHERWISE. OPINIONS EXPRESSED ARE OUR OWN UNLESS OTHERWISE STATED. PRICES SHOWN ARE APPROXIMATE.

THIS MATERIAL HAS BEEN APPROVED FOR DISTRIBUTION IN THE UNITED KINGDOM BY WILLIAM BLAIR INTERNATIONAL, LIMITED, REGULATED BY THE FINANCIAL CONDUCT AUTHORITY (FCA), AND IS DIRECTED ONLY AT, AND IS ONLY MADE AVAILABLE TO, PERSONS FALLING WITHIN COB 3.5 AND 3.6 OF THE FCA HANDBOOK (BEING “ELIGIBLE COUNTERPARTIES” AND “PROFESSIONAL CLIENTS”). THIS DOCUMENT IS NOT TO BE DISTRIBUTED OR PASSED ON TO ANY “RETAIL CLIENTS.” NO PERSONS OTHER THAN PERSONS TO WHOM THIS DOCUMENT IS DIRECTED SHOULD RELY ON IT OR ITS CONTENTS OR USE IT AS THE BASIS TO MAKE AN INVESTMENT DECISION.

“William Blair” and “R\*Docs” are registered trademarks of William Blair & Company, L.L.C. Copyright 2014, William Blair & Company, L.L.C. All rights reserved.