Portola Investor Room

Portola Pharmaceuticals Reports Fourth Quarter and Full Year 2017 Financial Results and Provides Corporate Update

--Conference Call Today at 4:30 p.m. ET--

SOUTH SAN FRANCISCO, Calif., Feb. 28, 2018 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals, Inc.® (NASDAQ:PTLA) today reported financial results for the fourth quarter and full year ended December 31, 2017 and provided a corporate update.

“2017 was a year of significant achievement for Portola, highlighted by the FDA approval of our first medicine, Bevyxxa, and the regulatory application for FDA approval of our breakthrough-designated Factor Xa-inhibitor antidote, AndexXa,” said Bill Lis, chief executive officer of Portola. “We are pleased with the early indicators of the U.S. commercial launch of Bevyxxa, which began in January, and the potential to have a major public health impact on millions of patients in the U.S. and beyond. We also are rapidly approaching the FDA action date for AndexXa, which, if approved, would position Portola with two first-in-class medicines in the field of thrombosis. In the meantime, we are working with European regulatory authorities on the path forward for both medicines and are continuing to advance our dual, oral Syk/JAK inhibitor, cerdulatinib, for hematologic cancers.”

Recent Achievements, Upcoming Events and Milestones

Bevyxxa® (betrixaban) – an oral, once-daily Factor Xa inhibitor approved for extended prophylaxis of venous thromboembolism (VTE) in acute medically ill patients with risk factors for VTE.

- Initiated U.S. commercial launch of Bevyxxa in January 2018
- Received FDA approval for the manufacturing of betrixaban in Cork, Ireland
- Completed an oral explanation to the European Committee for Medicinal Products for Human Use (CHMP); determining next steps in light of negative CHMP trend vote
- New APEX trial results published in *The American Heart Journal* showing betrixaban’s effect on symptomatic VTE and VTE-related deaths; 12th major peer-reviewed publication

AndexXa® (andexanet alfa) – an antidote for Factor Xa inhibitor treated patients with life-threatening bleeding; designated a Breakthrough Therapy and an Orphan Drug by the FDA.

- The FDA set a new action date of May 4, 2018 for the Biologics License Application (BLA)
- Successfully completed the first commercial campaign for Gen 2 product
- Enrollment remains on track for the ongoing Phase 3b/4 ANNEXA-4 study in patients with acute major bleeding
- Late-breaking clinical trial presentation on March 12, 2018 at the American College of Cardiology’s 67th Annual Scientific Session & Expo (ACC.18) featuring new interim data from ANNEXA-4
- Successfully completed an oral explanation to the European CHMP; received a positive CHMP trend vote and are working with regulatory authorities to address their accompanying request for additional data

Cerdulatinib – an oral, dual-spleen tyrosine kinase (Syk) and janus kinase (JAK) inhibitor in development for the treatment of relapsed/refractory B-cell and other T-cell malignancies in patients who have failed multiple therapies.

- Continued to enroll patients in a Phase 2a study evaluating the safety and efficacy of cerdulatinib in patients with relapsed/refractory B-cell and T-cell malignancies who have failed multiple therapies

Fourth Quarter and Full Year 2017 Financial Results

Collaboration and license revenue earned under Portola’s collaboration and license agreements with Bristol-Myers Squibb Company, Pfizer, Bayer Pharma, Janssen Pharmaceuticals, Daiichi Sankyo and Dermavant

Sciences was $9.8 million for the fourth quarter of 2017, compared with $13.7 million for the fourth quarter of 2016. Collaboration and license revenue for the year ended December 31, 2017 was $22.5 million, compared with $35.5 million for the year ended December 31, 2016.

Total operating expenses for the fourth quarter of 2017 were $95.7 million, compared with $68.9 million for the same period in 2016. Total operating expenses for the fourth quarter of 2017 included $10.9 million in stock-based compensation expense, compared with $7.9 million for the same period in 2016. Total operating expenses for the year ended December 31, 2017 were $295.2 million, compared with $305.1 million for 2016. Total operating expenses for the full year ended December 31, 2017 included $43.3 million in stock-based compensation expense compared with $30.4 million for 2016.

Research and development expenses were $68.5 million for the fourth quarter of 2017, compared with $56.0 million for the fourth quarter of 2016. Research and development expenses were $203.7 million for the year ended December 31, 2017, compared with $246.9 million for 2016.

Selling, general and administrative expenses for the fourth quarter of 2017 were $26.9 million, compared with $12.9 million for the same period in 2016. Selling, general and administrative expenses for the year ended December 31, 2017 were $91.1 million, compared with $58.2 million for 2016.

For the fourth quarter of 2017, Portola reported a net loss of $91.8 million, or $1.41 net loss per share, compared with a net loss of $53.8 million, or $0.95 net loss per share, for the same period in 2016. Shares used to compute net loss per share attributable to common stockholders were $65.3 million for the fourth quarter of 2017 compared with $56.5 million for the same period in 2016. Net loss for the year ended December 31, 2017 was $286.1 million, or $4.81 net loss per share, compared with a net loss of $269.0 million, or $4.76 net loss per share, for the same period in 2016. Shares used to compute net loss per share attributable to common stockholders were $59.5 million for 2017 compared with $56.5 million for 2016.

Cash, cash equivalents and investments at December 31, 2017 totaled $534.2 million, compared with cash, cash equivalents and investments of $318.8 million as of December 31, 2016.

If the FDA approves andexanet alfa in Q2 2018, the Company will be entitled to receive an additional $100 million from its royalty-based financing with Health Care Royalty Partners.

2018 Annual Financial Guidance

For the fiscal year 2018, Portola expects total GAAP operating expenses to be between $390 million and $430 million, including stock based compensation. These expenses are primarily for manufacturing of both andexanet alfa and betrixaban, ongoing clinical trials, support for the commercial launch of Bevyxxa and preparation for the potential commercial launch of AndexXa.

Conference Call Details

Portola will host a conference call today, Wednesday, February 28, 2018, at 4:30 p.m. ET, during which time management will provide fourth quarter and full year 2017 financial results, updates on the U.S. launch of Bevyxxa and other matters. The live call can be accessed by phone by dialing (844) 452-6828 from the United States and Canada or 1 (765) 507-2588 internationally and using the passcode 4893459. The webcast can be accessed live on the Investor Relations section of the Company's website at http://investors.portola.com. It will be archived for 30 days following the call.

About Portola Pharmaceuticals, Inc.

Portola Pharmaceuticals is a biopharmaceutical company developing product candidates that could significantly advance the fields of thrombosis and other hematologic diseases. The Company’s first medicine Bevyxxa® (betrixaban), an oral, once-daily Factor Xa inhibitor, was approved by the U.S. Food and Drug Administration in June 2017. The company is also working to advance two clinical programs for andexanet alfa, a recombinant protein designed to reverse the anticoagulant effect in patients treated with an oral or injectable Factor Xa inhibitor; and cerdulatinib, a SYK/JAK inhibitor in development to treat hematologic cancers. Portola's

partnered program is focused on developing selective SYK inhibitors for inflammatory conditions. For more information, visit http://www.portola.com and follow the Company on Twitter @Portola_Pharma.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, statements regarding the potential public health impact and commercial success of Bevyxxa, regulatory approval of andexanet alfa, projected operating expenses for 2018 and cerdulatinib's potential as a treatment for hematologic cancers. Risks that contribute to the uncertain nature of the forward-looking statements include: failure to obtain FDA and/or EMA approval for one or more of our product candidates, regulatory developments in the United States and foreign countries; our expectation that we will incur losses for the foreseeable future and will need additional funds to finance our operations; the accuracy of our estimates regarding our ability to initiate and/or complete our clinical trials and the timing and expense of these trials; the results of our clinical trials related to the efficacy and safety of our product candidates; our potential inability to manufacture our product candidates on a commercial scale in a timely or cost-efficient manner; the accuracy of our estimates regarding expenses and capital requirements; our ability to successfully build a hospital-based sales force and commercial infrastructure; our ability to obtain and maintain intellectual property protection for our product candidates; and our ability to retain key scientific or management personnel. These and other risks and uncertainties are described more fully in our most recent filings with the Securities and Exchange Commission, including our most recent quarterly report on Form 10-Q. All forward-looking statements contained in this press release speak only as of the date on which they were made. We undertake no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

Investor Contact:
Cara Miller
Portola Pharmaceuticals
ir@portola.com

Media Contact:
Laurie Masonson
W2O Group
lmasonson@w2ogroup.com

Unaudited Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
Collaboration and license revenue	$ 9,803	$ 13,693	$ 22,546	$ 35,504
Operating expenses:				
Cost of sales	260	—	415	—
Research and development	68,491	56,032	203,701	246,854
Selling, general and administrative	26,903	12,861	91,109	58,235
Total operating expenses	95,654	68,893	295,225	305,089
Loss from operations	(85,851)	(55,200)	(272,679)	(269,585)
Interest and other income (expense), net	(2,290)	497	(1,338)	1,533
Interest expense	(3,360)	(61)	(11,603)	(61)
Loss before taxes	(91,501)	(54,764)	(285,620)	(268,113)
Income tax benefit	—	—	—	—
Net loss	(91,501)	(54,764)	(285,620)	(268,113)

Net loss (income) attributable to noncontrolling interest (SRX Cardio)	(280)	923	(470)	(930)
Net loss attributable to Portola	$ (91,781)	$ (53,841)	$ (286,090)	$ (269,043)
Net loss per share attributable to Portola common stockholders:				
Basic and diluted	$ (1.41)	$ (0.95)	$ (4.81)	$ (4.76)
Shares used to compute net loss per share attributable to Portola common stockholders:				
Basic and diluted	65,260,653	56,543,875	59,508,156	56,480,647

Consolidated Balance Sheet Data

(In thousands)

	December 31, 2017 (Unaudited)	December 31, 2016
Cash, cash equivalents and investments	$ 534,233	$ 318,771
Receivables from collaborators	3,750	—
Prepaid research and development	734	7,299
Total current assets	477,923	328,928
Property and equipment, net	5,217	6,143
Intangible assets	7,851	3,151
Prepaid and other long-term assets	9,609	5,214
Total assets	571,676	343,436
Accounts payable	9,304	14,546
Accrued research and development	44,973	23,818
Accrued compensation and other liabilities	15,078	6,502
Deferred revenue (current portion and long-term)	29,967	45,763
Total current liabilities	80,524	65,664
Long term obligation to Collaborator	8,000	8,000
Notes payable, long-term and Long-term debt	104,816	49,815
Total liabilities	222,183	150,747
Total Portola stockholders’ equity	346,866	190,532
Noncontrolling interest (SRX Cardio)	2,627	2,157
Total stockholders' equity	349,493	192,689
Total liabilities and stockholders’ equity	571,676	343,436

Portola Pharmaceuticals, Inc.

https://investors.portola.com/2018-02-28-Portola-Pharmaceuticals-Reports-Fourth-Quarter-and-Full-Year-2017-Financial-Results-and-Provides-Corporate-Update