Portola Investor Room

Portola Pharmaceuticals Reports Second Quarter 2017 Financial Results and Provides Corporate Update

SOUTH SAN FRANCISCO, Calif., Aug. 09, 2017 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals Inc.® (Nasdaq:PTLA) today reported financial results and provided a corporate update for the quarter ended June 30, 2017.

“FDA approval of our first product, Bevyxxa®, in the second quarter marked the ultimate milestone for Portola and the millions of patients who could benefit from this important new medicine,” said Bill Lis, chief executive officer of Portola. “We resubmitted our BLA for AndexXa® in the U.S. and are committed to working closely with the FDA toward approval, and with the EMA for approval of both products in 2018. Based on robust clinical data, both Bevyxxa and AndexXa are potentially life-saving medicines and are highly anticipated by the medical community.”

Recent Achievements, Upcoming Events and Milestones

Bevyxxa (betrixaban) -– an oral, once-daily Factor Xa inhibitor approved by the U.S. Food and Drug Administration (FDA) under Priority Review on June 23, 2017

- First and only anticoagulant for hospital and extended duration prophylaxis (35 to 42 days) of venous thromboembolism (VTE) in adult patients hospitalized for an acute medical illness who are at risk for thromboembolic complications due to moderate or severe restricted mobility and other risk factors for VTE
- Anticipate U.S. launch between September and November 2017
- Expect opinion from the Committee for Medicinal Products for Human Use (CHMP) by late 2017 or early 2018

AndexXa (andexanet alfa) – a Factor Xa inhibitor antidote in development for patients treated with a Factor Xa inhibitor when reversal of anticoagulation is needed due to life-threatening bleeding or when urgent surgery is required; designated a Breakthrough Therapy and an Orphan Drug by the FDA

- Resubmitted Biologics License Application (BLA) to the FDA on August 3, 2017
- Expect to receive an opinion from the CHMP by early 2018

Cerdulatinib – an oral, dual Syk/JAK inhibitor in development to treat relapsed and refractory hematologic cancers

- Presented interim data at the International Congress of Malignant Lymphoma and the European Hematology Association from a Phase 2a study evaluating cerdulatnib in patients with relapsed/refractory B-cell malignancies that demonstrated evidence of clinical activity in patients with relapsed/refractory B-cell malignancies; also presented preliminary data suggesting activity in t-cell lymphoma

Second Quarter 2017 Financial Results
Collaboration and license revenue earned under Portola's collaboration and license agreements with Bristol-Myers Squibb Company and Pfizer, Bayer Pharma, Janssen Pharmaceuticals and Daiichi Sankyo was $3.8 million for the second quarter of 2017 compared with $4.2 million for the second quarter of 2016.

Total operating expenses for the second quarter of 2017 were $69.6 million, compared with $61.9 million for the same period in 2016. Total operating expenses for the second quarter of 2017 included $13.3 million in stock-based compensation expense, compared with $7.6 million for the same period in 2016.

Research and development expenses were $49.3 million for the second quarter of 2017, compared with $44.8 million for the second quarter of 2016. The increase in R&D expenses was largely attributable to an increase in manufacturing costs to produce betrixaban active pharmaceutical ingredient and other program costs related

to cerdulatinib.

Selling, general and administrative expenses for the second quarter of 2017 were $20.3 million, compared with $17.0 million for the same period in 2016.

For the second quarter of 2017, Portola reported a net loss of $69.7 million, or $1.22 net loss per share, compared with a net loss of $57.3 million, or $1.02 net loss per share, for the same period in 2016.

Cash, cash equivalents and investments at June 30, 2017 totaled $269.7 million, compared with cash, cash equivalents and investments of $318.8 million as of December 31, 2016.

Conference Call Details
Portola will host a conference call today, Wednesday, August 9, 2017, at 4:30 p.m. Eastern Time, during which management will discuss the remaining steps necessary for the planned U.S. launch of Bevyxxa, second quarter 2017 financial results and other matters. The live call can be accessed by phone by calling (844) 452-6828 from the United States and Canada or 1 (765)-507-2588 internationally and using the passcode 61767329. 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 AndexXa™ (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, anticipated regulatory actions by the FDA and EMA and the potential of Bevyxxa and Portola’s other product candidates. 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration and license revenue	$ 3,787	$ 4,231	$ 8,915	$ 12,489
Operating expenses:				
Research and development	49,292	44,823	79,937	103,636
Selling, general and administrative	20,329	17,044	35,350	31,795
Total operating expenses	69,621	61,867	115,287	135,431
Loss from operations	(65,834)	(57,636)	(106,372)	(122,942)
Interest and other income (expense), net	(124)	297	289	629
Interest expense	(3,456)	—	(5,095)	—
Net loss	(69,414)	(57,339)	(111,178)	(122,313)
Net income attributable to noncontrolling interest (SRX Cardio)	(240)	—	(195)	—
Net loss attributable to Portola	$ (69,654)	$ (57,339)	$ (111,373)	$ (122,313)
Net loss per share attributable to Portola common stockholders:				
Basic and diluted	$ (1.22)	$ (1.02)	$ (1.96)	$ (2.17)
Shares used to compute net loss per share attributable to Portola common stockholders:				
Basic and diluted	57,050,523	56,399,535	56,872,644	56,434,644

Unaudited Condensed Consolidated Balance Sheet Data

(In thousands)

	June 30, 2017 (Unaudited)	December 31, 2016
Cash, cash equivalents and investments	$ 269,698	$ 318,771
Prepaid research and development	3,487	7,299
Total current assets	285,687	328,928
Property and equipment, net	5,576	6,143
Intangible asset	8,151	3,151
Prepaid and other long-term assets	6,277	5,214
Total assets	305,691	343,436
Accounts payable	11,938	14,546
Accrued research and development	22,050	23,818
Accrued compensation and other liabilities	8,598	6,502
Deferred revenue (current portion and long-term)	36,848	45,763
Total current liabilities	57,773	65,664
Notes payable, long-term	51,627	49,815
Long term debt	50,117	—
Long term obligation to Collaborator	8,000	8,000
Total liabilities	192,324	150,747
Total Portola stockholders’ equity	111,015	190,532
Noncontrolling interest (SRX Cardio)	2,352	2,157
Total stockholders’ equity	113,367	192,689
Total liabilities and stockholders’ equity	305,691	343,436

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