Portola Investor Room

Portola Pharmaceuticals Reports First Quarter 2018 Financial Results and Provides Corporate Update

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

SOUTH SAN FRANCISCO, Calif., May 09, 2018 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals, Inc.® (Nasdaq:PTLA) today reported financial results for the three months ended March 31, 2018 and provided a corporate update.

"We achieved several major manufacturing and regulatory milestones this past year, including last week's U.S. approval of Andexxa®. We are now concentrating the Company's efforts on the successful launch of Andexxa and Bevyxxa®, which both have the potential to impact public health and become standards of care in the field of thrombosis," said Bill Lis, chief executive officer of Portola. "We are also encouraged by interim data for our next compound for hematologic cancers, cerdulatinib, which will be presented next month at the American Society of Clinical Oncology Annual Meeting. Together, these three compounds comprise a leading thrombosis and hematology portfolio, all with global rights."

Recent Achievements, Upcoming Events and Milestones

Andexxa [coagulation factor Xa (recombinant), inactivated-zhzo]– antidote for the reversal of the Factor Xa inhibitors rivaroxaban and apixaban.

- Andexxa received Accelerated Approval from the FDA on May 3, 2018 for patients treated with rivaroxaban and apixaban, when reversal of anticoagulation is needed due to life-threatening or uncontrolled bleeding.
- Earned an additional $100 million milestone payment from the Company's royalty-based financing with Health Care Royalty Partners based on the FDA approval of Andexxa in May 2018.
- Early Supply Program to launch in June with broader commercial launch anticipated in early 2019, upon FDA approval of the Generation 2 product.
- Presented interim data from the ongoing Phase 3b/4 ANNEXA-4 study in a late-breaking clinical trial presentation at the American College of Cardiology's 67th Annual Scientific Session & Expo (ACC.18). Enrollment on track for completion this summer.
- Received a positive CHMP trend vote and working with regulatory authorities to address their accompanying request for additional data.
- Built significant Generation 2 product inventory to meet broad demand upon regulatory approval in the U.S. and Europe.

Bevyxxa (betrixaban) – 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 and continued to expand the field force and market access teams.

- Additional results from the APEX trial published in the *American Heart Journal* and the *American Journal of Medicine* continue to highlight betrixaban's effect on symptomatic VTE and VTE-related deaths.
- Eight abstracts accepted at the upcoming International Society on Thrombosis and Haemostasis (ISTH) and European Society of Cardiology (ESC) meetings.

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.

- Completed enrollment in two of four cohorts of the ongoing 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.
- Interim results from the ongoing Phase 2a study accepted for presentation at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting.
- Received initial feedback from the FDA on the potential regulatory pathway.

First Quarter 2018 Financial Results

Total revenue for the first quarter of 2018 was $6.6 million, compared with $5.1 million for the first quarter of 2017. This includes $6.0 million in collaboration and license revenue earned under Portola's collaboration and license agreements with Bristol-Myers Squibb Company, Pfizer, Bayer Pharma, Janssen Pharmaceuticals and Daiichi Sankyo, as well as $0.6 million in product revenue from initial sales of Bevyxxa, which was launched in the U.S. in January 2018.

Total operating expenses for the first quarter of 2018 were $91.9 million, compared with $45.7 million for the same period in 2017. Total operating expenses for the first quarter of 2018 included $11.0 million in stock-based compensation expense, compared with $9.0 million for the same period in 2017.

Research and development expenses were $60.1 million for the first quarter of 2018, compared with $30.6 million for the first quarter of 2017. The increase is due to the second Generation 2 commercial manufacturing campaign. Selling, general and administrative expenses for the first quarter of 2018 were $31.5 million, compared with $15.0 million for the same period in 2017. The increase is due to the build-out of the field force and marketing spend for the Bevyxxa launch.

For the first quarter of 2018, Portola reported a net loss of $84.2 million, or $1.28 net loss per share, compared with a net loss of $41.7 million, or $0.74 net loss per share, for the same period in 2017. Shares used to compute net loss per share attributable to common stockholders were 65.5 million for the first quarter of 2018 compared with 56.7 million for the same period in 2017.

Cash, cash equivalents and investments at March 31, 2018 totaled $451.1 million, compared with cash, cash equivalents and investments of $534.2 million as of December 31, 2017.

Based on the FDA approval of Andexxa in May 2018, the Company earned an additional $100 million milestone payment from its royalty-based financing with Health Care Royalty Partners.

Conference Call Details

Portola will host a conference call today, Wednesday, May 9, 2018, at 4:30 p.m. ET, during which time management will provide first quarter 2018 financial results, updates on Andexxa, the U.S. launch of Bevyxxa and other matters. The live call can be accessed by phone by dialing (844) 452-6828 from the U.S. and Canada or 1 (765) 507-2588 internationally and using the passcode 7068059. 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 commercial-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics that could significantly advance the fields of thrombosis and other hematologic diseases. The Company's two FDA-approved medicines are Bevyxxa® (betrixaban), the first and only oral, once-daily Factor Xa inhibitor, and Andexxa® [coagulation factor Xa (recombinant), inactivated-zhzo], the first and only antidote for the Factor Xa inhibitors rivaroxaban and apixaban. The company also is advancing cerdulatinib, a SYK/JAK inhibitor for the treatment of hematologic cancers.

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 of Andexxa and Bevyxxa, the timing of the anticipated Early Supply Program launch of Andexxa, our plans to present cerdulatinib interim Phase 2a clinical trial results and our expected receipt of an additional $100 million in royalty-based financing investment. Risks that contribute to the uncertain nature of the forward-looking statements include: the risk that physicians, patients and payers may not see the benefits of utilizing Andexxa or Bevyxxa for the indications which they are approved; our ability to continue to manufacture our products and to expand approved manufacturing facilities; the possibility of unfavorable results from additional clinical trials involving Andexxa; the risk that the EMA may not approve Andexxa in the currently anticipated timelines or at all, and that any marketing approvals or reimbursement limitations may have significant limitations on its use; the risk that Portola may not obtain additional regulatory approvals necessary to expand approved indications for Andexxa; 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 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.

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

	Three Months Ended March 31,	
	2018	**2017**
Revenues:		
Product revenue, net	$ 606	$ —
Collaboration and license revenue	6,038	5,128
Total revenues	6,644	5,128
Operating expenses:		
Cost of Sales	336	—
Research and development	60,067	30,645
Selling, general and administrative	31,541	15,021
Total operating expenses	91,944	45,666
Loss from operations	(85,300)	(40,538)
Interest and other income, net	3,371	413
Interest expense	(2,581)	(1,639)
Net loss	(84,510)	(41,764)
Net loss attributable to noncontrolling interest (SRX Cardio)	332	45
Net loss attributable to Portola	$ (84,178)	$ (41,719)
Net loss per share attributable to Portola common stockholders:		
Basic and diluted	$ (1.28)	$ (0.74)
Shares used to compute net loss per share attributable to Portola common stockholders:		
Basic and diluted	65,509,945	56,692,788

Unaudited Condensed Consolidated Balance Sheet Data

(In thousands)

	March 31, 2018 (Unaudited)	December 31, 2017
Cash, cash equivalents and investments	$ 451,085	$ 534,233
Prepaid research and development	3,449	734
Trade and other receivables, net	1,693	3,750
Unbilled - collaboration and license revenue	4,660	—
Total current assets	436,237	477,923
Property and equipment, net	5,393	5,217
Intangible assets	7,710	7,851
Prepaid and other long-term assets	4,812	9,609
Total assets	496,666	571,676
Accounts payable	20,788	9,304
Accrued research and development	22,105	44,973
Accrued compensation and other liabilities	10,754	15,078
Deferred revenue (current portion and long-term)	9,273	29,967
Total current liabilities	57,508	80,524
Notes payable, long-term and Long-term debt	107,397	104,816
Long term obligation to Collaborator	8,000	8,000
Total liabilities	186,498	222,183
Total Portola stockholders' equity	307,992	346,866
Noncontrolling interest (SRX Cardio)	2,176	2,627
Total stockholders' equity	310,168	349,493
Total liabilities and stockholders' equity	496,666	571,676

Source: Portola Pharmaceuticals, Inc.

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