



PORTOLA[®]
PHARMACEUTICALS

Portola Pharmaceuticals Reports Second Quarter 2019 Financial Results and Provides Corporate Update

August 7, 2019

- **Second Quarter Revenues of \$28.4 Million; Andexxa[®] Net Product Revenues Grow to \$27.1 Million -**
- **Launched Ondexxya[®] (andexanet alfa) with First Orders in Europe -**
- **U.S. Centers for Medicare and Medicaid Services Increased New Technology Add-On Payment (NTAP) for Andexxa from 50 to 65 Percent -**
- **Conference Call Today at 1:30 p.m. PT / 4:30 p.m. ET -**

SOUTH SAN FRANCISCO, Calif., Aug. 7, 2019 /PRNewswire/ -- Portola Pharmaceuticals, Inc.[®] (Nasdaq: PTLA) today reported financial results for the three months ended June 30, 2019, and provided a corporate update.



"This is our fifth consecutive quarter of strong revenue growth reflecting our exceptional launch execution and continued demand for Andexxa. Support for this novel therapy continues to grow with CMS' decision to increase our NTAP reimbursement and two recent updates from the Joint Commission which recommend specific reversal agents for Factor Xa inhibitors. In Europe, the team exceeded expectations on timing for the first sale of Ondexxya, and we are positioned well to continue our launch in key European countries," said Scott Garland, Portola's president and chief executive officer. "We look forward to building upon our momentum backed by a rapidly growing Factor Xa inhibitor market and increasing global demand for Andexxa. Beyond Andexxa, we plan to initiate a registrational trial for cerdulatinib."

Quarter Ending June 30, 2019

- Total revenues for the second quarter of 2019 were \$28.4 million, compared with \$4.0 million for the second quarter of 2018. This includes \$27.1 million in net product revenues from sales of Andexxa [coagulation factor Xa (recombinant), inactivated-zhzo], \$74 thousand in revenues from Bevyxxa[®] (betrixaban) sales and \$1.3 million in collaboration and license revenues.
- Net loss attributable to Portola, according to generally accepted accounting principles in the U.S. (GAAP), was \$66.2 million, or \$0.97 net loss per share for the second quarter of 2019, compared with a net loss of \$106.2 million, or \$1.61 net loss per share, for the same period in 2018. This includes the effect of a \$3.1 million impairment charge taken in the second quarter related to the discontinuation of our SRX program.
- Cash, cash equivalents and investments at June 30, 2019, totaled \$273.9 million, compared with \$317.0 million as of December 31, 2018.
- Total GAAP operating expenses for the second quarter of 2019 were \$92.4 million, compared with \$107.7 million for the same period in 2018. This decrease was driven primarily by manufacturing costs for Andexxa Gen 2 being capitalized and no longer flowing through R&D.
- Stock-based compensation expense for the second quarter of 2019 was \$12.3 million, compared with \$13.2 million for the same period in 2018.

- Cost of Sales (COS) for the second quarter of 2019 was \$5.0 million, compared to \$1.1 million for the same period in 2018. The increase was driven by the launch of Andexxa.
- Research and development (R&D) expenses were \$33.5 million for the second quarter of 2019, which includes the impairment charge, compared with \$66.4 million for the second quarter of 2018. The decrease was driven primarily by the manufacturing costs for Andexxa Gen 2 being capitalized and no longer flowing through R&D and partially offset by the SRX program impairment charge.
- Non-GAAP research and development expenses, which excludes the SRX program impairment charge, were \$30.4 million for the second quarter of 2019. Please see the reconciliation of GAAP to non-GAAP financial measures table at the end of this release for more details.
- Selling, general and administrative (SG&A) expenses for the second quarter of 2019 were \$53.9 million, compared with \$40.2 million for the same period in 2018. The increase was driven by the expansion of the Company's field force, commercial activities to support the launch of Andexxa and launch preparations in Europe.

Recent Achievements and Events

- Launched Ondexxya with first orders in Europe.
- CMS increased maximum NTAP reimbursement for Andexxa from 50 to 65 percent effective on October 1, 2019.
- Presented new Andexxa data from a subset of patients from the ANNEXA-4 study with spontaneous (non-traumatic) intracranial hemorrhage, which demonstrated excellent or good hemostasis achieved in 79% of evaluable patients.
- Presented in vitro data demonstrating that four-factor prothrombin complex concentrate (4F-PCC) does not appear to have an effect on the inhibition of thrombin generation by apixaban or rivaroxaban unless the Factor Xa inhibitor concentration was less than 75 ng/mL. In contrast, data from the same thrombin generation assay demonstrated that Andexxa fully corrected the inhibition of thrombin generation by apixaban and rivaroxaban across a broad range of inhibitor concentrations.
- Presented new interim results from the cerdulatinib Phase 2a study demonstrating favorable safety and efficacy profiles in patients with relapsed/refractory follicular lymphoma (FL) receiving cerdulatinib alone (45% objective response rate) or in combination with rituximab (62% objective response rate).

Planned Upcoming Milestones

- Continue launch of Ondexxya in a select group of high-potential European countries where Factor Xa inhibitor use is among the highest.
- Plan to initiate surgical study for Andexxa label expansion by year end or beginning of 2020.
- Launch a cerdulatinib registrational study in peripheral T-cell lymphoma (PTCL) by the end of the year.
- Present new data from additional subsets of the ANNEXA-4 study.

Conference Call Details

Portola will host a conference call today, Wednesday, August 7, 2019, at 4:30 p.m. ET, during which time management will discuss the second quarter 2019 financial results, updates on the U.S. launch of Andexxa, 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 8046269. 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.

Use of Non-GAAP Financial Measures

This press release and the reconciliation table included herein include non-GAAP R&D expenses. The Company believes the presentation of non-GAAP financial measures provides useful information to management and investors regarding the company's financial condition and results of operations. When viewed in conjunction with GAAP financial measures, investors are provided with a more meaningful understanding of the Company's ongoing operating performance and are better able to compare the Company's performance between periods. In addition, these non-GAAP financial measures are among those that the Company uses as a basis for evaluating performance, allocating resources and planning and forecasting future periods. Non-GAAP financial measures are not intended to be considered in isolation or as a substitute for GAAP financial measures. A reconciliation of GAAP to non-GAAP financial measures is provided in the accompanying table entitled "Reconciliation of GAAP to Non-GAAP Financial Information."

Unaudited Condensed Consolidated Statements of Operations

(In thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenues:				
Product revenue, net	\$ 27,164	\$ 2,265	\$ 47,526	\$ 2,871
Collaboration and license revenue	1,260	1,746	3,067	7,784
Total revenues	28,424	4,011	50,593	10,655
Operating expenses:				

Cost of Sales	4,991	1,052	12,141	1,388
Research and development	33,538	66,440	69,122	126,507
Selling, general and administrative	53,855	40,214	106,889	71,755
Total operating expenses	92,384	107,706	188,152	199,650
Loss from operations	(63,960)	(103,695)	(137,559)	(188,995)
Interest and other income, net	4,021	1,828	6,005	5,199
Interest expense	(8,538)	(4,104)	(15,019)	(6,685)
Net loss	(68,477)	(105,971)	(146,573)	(190,481)
Net (income) loss attributable to noncontrolling interest	2,273	(223)	2,213	109
Net loss attributable to Portola	\$ (66,204)	\$ (106,194)	\$ (144,360)	\$ (190,372)
Net loss per share attributable to Portola common stockholders:				
Basic and diluted	\$ (0.97)	\$ (1.61)	\$ (2.14)	\$ (2.90)
Shares used to compute net loss per share attributable to Portola common stockholders:				
Basic and diluted	68,128,238	65,884,767	67,602,126	65,698,391

Unaudited Condensed Consolidated Balance Sheet Data

(In thousands)

	June 30, 2019	December 31, 2018
	(Unaudited)	
Cash, cash equivalents and investments	\$ 273,938	\$ 316,964
Trade and other receivables, net	13,955	5,849
Unbilled - collaboration and license revenue	4,795	9,880
Inventories	1,547	7,873
Property and equipment, net	4,769	5,236
Intangible assets	3,844	7,279
Other assets	63,649	33,338
Total assets	366,497	386,419
Current liabilities	84,307	69,005
Long-term liabilities	281,596	226,847
Total stockholders' equity	594	90,567
Total liabilities and stockholders' equity	366,497	386,419

PORTOLA PHARMACEUTICALS, INC.

Reconciliation of GAAP to Non-GAAP Financial Information

(In thousands)

	Three Months Ended June 30, 2019
Reconciliation of research and development:	
GAAP Research and development	33,538
SRX intangible impairment	(3,151)
Non-GAAP research and development expenses	\$ 30,387

About Portola Pharmaceuticals, Inc.

Portola Pharmaceuticals is a global, 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 conditions. The Company's first two commercialized products are Andexxa® [coagulation factor Xa (recombinant), inactivated-zhzo], marketed in Europe as Ondexxya® (andexanet alfa), and Bevyxxa® (betrixaban). The company also is advancing cerdulatinib, a SYK/JAK inhibitor being developed for the treatment of hematologic cancers. Founded in 2003 in South San Francisco, California, Portola has operations in the United States and Europe.

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 anticipated initiation of a registrational trial for cerdulatinib in PTCL, the growing demand for our products, our continued commercial launch for Ondexxya in Europe, potential label expansion opportunities for Andexxa and our plans to present new data and continue development of our products and product candidates. 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 for the indications which it is 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; our ability to grow our commercial operations in the EU and generate product revenue within projected timelines and budget; the risk that we 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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