European CHMP Maintains Negative Opinion on Portola Pharmaceuticals' Betrixaban Following Appeal

SOUTH SAN FRANCISCO, Calif., July 27, 2018 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals, Inc.® (Nasdaq:PTLA) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a final recommendation on betrixaban for the prevention of venous thromboembolism (VTE) in adult patients hospitalized for an acute medical illness with risk factors for VTE, including VTE-related death. The CHMP maintained its negative opinion following a re-examination procedure requested by the Company.

The CHMP's position is that betrixaban addresses an unmet medical need and showed a clinically relevant rate of prevention of thrombotic events in patients treated with betrixaban compared to those treated with enoxaparin. However, the committee did not consider the overall risk-benefit profile sufficiently robust when considering non-major bleeding in addition to major bleeding. Portola will continue working with regulatory authorities in countries outside the European Union to potentially bring this important new treatment option to patients as quickly as possible.

"We are disappointed by the Committee's assessment given both the unmet need and the clinically meaningful outcomes of the APEX trial in reducing VTE and VTE-related deaths in a vulnerable patient population," said John T. Curnutte, M.D., Ph.D., interim co-president and head of research and development for Portola. "We remain confident in the potential public health impact of betrixaban and will maintain focus on the U.S. commercial launch as we continue to work toward our goal of expanding patient access to betrixaban around the world."

The Marketing Authorization Application (MAA) for betrixaban included data from Portola's pivotal Phase 3 APEX Study, which enrolled 7,513 patients at more than 450 clinical sites worldwide. The APEX study evaluated oral betrixaban from hospital admission to home (35 to 42 days) compared with injectable enoxaparin for 6 to 14 days followed by placebo in assessing the prevention of VTE in high-risk acutely ill medical patients.

Betrixaban was approved by the U.S. Food and Drug Administration in June 2017 under the trade name Bevyxxa®.

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 for the prevention of VTE in adult patients hospitalized for an acute medical illness, 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

This announcement contains forward-looking statements, including statements relating to Portola Pharmaceuticals' expectations regarding the regulatory and development status of betrixaban. These statements are subject to significant risks and uncertainties, and actual results could differ materially from those projected. These risks and uncertainties include, without limitation, risks and uncertainties related to the regulatory and development process for betrixaban in Europe and other countries, risks and uncertainties that physicians may not see the benefits of utilizing betrixaban or Andexxa for the indications which they are approved; the ability of Portola to continue to manufacture our products and to expand approved manufacturing facilities; the possibility of unfavorable results from additional studies of betrixaban; the risk that the EMA may not approve Andexxa in the currently anticipated timelines or at all, and that any marketing approvals may have significant limitations on its use; the risk that Portola may not obtain additional regulatory approvals necessary to expand approved indications for Andexxa; and other general business risks which could have a material adverse impact on Portola's business, including risks associated with the launch of Portola's products; regulatory actions or delays or government regulation generally; Portola's ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures; general economic and industry conditions, including the effects of the persistently weak economic and financial environment in many countries; safety, quality or manufacturing issues. Risks and uncertainties relating to Portola Pharmaceuticals and its business can be found in the “Risk Factors” section of Portola Pharmaceuticals’ Annual Report on Form 10-K for 2017, which was filed with the SEC on March 1, 2018, as updated by subsequent periodic reports filed by Portola with the SEC, including Quarterly Reports on Form 10-Q and Current Reports on Form 8-K which are deemed “filed” with the SEC. Portola Pharmaceuticals undertakes no duty or obligation to update any forward-looking statements contained in this release as a result of new information, future events or changes in Portola Pharmaceuticals’ expectations.

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