Jazz Pharmaceuticals and Nippon Shinyaku Enter Into License Agreements for the Development and Commercialization of Defitelio and Vyxeos in Japan

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Jazz to receive an upfront payment and subsequent milestone-based payments

DUBLIN, March 30, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that it has entered into license agreements with Nippon Shinyaku, Co., Ltd. for Defitelio® (defibrotide sodium) and Vyxeos ™ (cytarabine and daunorubicin liposome injection), or CPX-351, in Japan.

Under the terms of the agreements, Nippon Shinyaku will receive exclusive rights to develop and commercialize Defitelio and Vyxeos in Japan in return for an upfront payment to Jazz Pharmaceuticals and subsequent payments based on the successful achievement of certain regulatory and commercial milestones. Jazz Pharmaceuticals will manufacture and supply Defitelio and Vyxeos to Nippon Shinyaku, and will receive revenue based on a percentage of product sales in Japan. Financial terms of the agreement have not been disclosed. Commercialization of Defitelio and Vyxeos in Japan is subject to regulatory approval in Japan.

"We are pleased to have Nippon Shinyaku as our strategic partner in Japan," said Iain McGill, senior vice president, Jazz Pharmaceuticals Europe and rest of world. "Nippon Shinyaku's expertise and focus in hematology/oncology make them an outstanding partner to bring Defitelio and Vyxeos to patients with significant unmet medical needs in Japan."

About Defitelio

Defitelio received marketing approval in the U.S. in 2016 for the treatment of adult and pediatric patients with hepatic veno-occlusive disease (VOD), with renal or pulmonary dysfunction following hematopoietic stem-cell transplantation (HSCT). In 2013, the European Commission granted marketing authorization to Defitelio under exceptional circumstances for the treatment of severe VOD in patients undergoing HSCT therapy. HSCT is an aggressive, potentially curative procedure to treat patients with malignant and non-cancerous hematologic disorders such as leukemia, lymphoma and aplastic anemia, and congenital immunodeficiency and autoimmune disorders. VOD is a rare and potentially life-threatening complication of HSCT affecting the sinusoidal endothelial cells of the liver, which occurs in approximately 9-14% of HSCT patients.[1,2] Defibrotide is also being investigated in a global Phase 3 randomized study (NCT02851407) for the prevention of hepatic VOD in high risk adult and pediatric patients undergoing HSCT. Defibrotide is currently an investigational drug in Japan and the Defitelio trade name has not been approved.

About Vyxeos (CPX-351)

Vyxeos, or CPX-351, is an investigational product being evaluated for the treatment of AML and is a combination of the antineoplastic agents cytarabine and daunorubicin encapsulated within a nano-scale liposome at a 5:1 molar ratio. The proposed trade name, Vyxeos ™, is conditionally approved by the U.S. Food and Drug Administration (FDA) and is subject to confirmation upon approval of the New Drug Application (NDA). Data from the pivotal Phase 3 study, which met its primary endpoint, were presented at the American Society of Clinical Oncology in June 2016. Jazz Pharmaceuticals has initiated a rolling NDA submission to the FDA with expected completion of the submission by the end of March 2017. A Marketing Authorization Application to the European Medicines Agency is planned for the second half of 2017. There have been no studies with CPX-351 in Japan, and the Vyxeos trade name has not been approved.

About Jazz Pharmaceuticals

Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is an international biopharmaceutical company focused on improving patients' lives by identifying, developing and commercializing meaningful products that address unmet medical needs. The company has a diverse portfolio of products and product candidates, with a focus in the areas of sleep and hematology/oncology. In these areas, Jazz Pharmaceuticals markets Xyrem® (sodium oxybate) oral solution, Erwinaze® (asparaginase *Erwinia chrysanthemi*) and Defitelio® (defibrotide sodium) in the U.S. and markets Erwinase® and Defitelio® (defibrotide) in countries outside the U.S. For more information, please visit www.jazzpharmaceuticals.com.

"Safe Harbor" Statement Under the Private Securities Litigation Reform Act of 1995

This press release contains forward-looking statements, including, but not limited to, statements related to the commercialization of Defitelio and Vyxeos in Japan, the expected timing of completion of submission of the NDA for Vyxeos to the FDA and the company's planned submission of an MAA for Vyxeos to the EMA. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with: pharmaceutical product development, including any additional development work required to support a regulatory filing for Defitelio and/or Vyxeos in Japan; and the regulatory approval process, including the risk that the company is unable to obtain regulatory approval for Defitelio and/or Vyxeos in Japan in a timely manner or at all; and other risks and uncertainties affecting the company and its development programs, including those described from time to time under the caption "Risk Factors" and elsewhere in Jazz Pharmaceuticals plc's Securities and Exchange Commission filings and reports (Commission File No. 001-33500), including the company's Annual Report on Form 10-K for the year ended December 31, 2016 and future filings and reports by the company. Other risks and uncertainties of which the company is not currently aware may also affect the company's forward-looking statements and may cause actual results and the timing of events to differ materially from those anticipated. The forward-looking statements herein are made only as of the date hereof or as of the dates indicated in the forward-looking statements, even if they are subsequently made available by the company on its website or otherwise. The company undertakes no obligation to update or supplement any forward-looking statements to reflect actual results, new information, future events, changes in its expectations or other circumstances that exist after the date as of which the forward-looking statements were made.

References:

1 Coppell JA, Richardson PG, Soiffer R, et al. Hepatic veno-occlusive disease following stem cell transplantation: incidence, clinical course, and outcome. Biol Blood Marrow Transplant. 2010;16(2):157-168.

2 Tsirigotis PD, Resnick IB, Avni B, et al. Incidence and risk factors for moderate-to-severe veno-occlusive disease of the liver after allogeneic stem cell transplantation using a reduced intensity conditioning regimen. Bone Marrow Transplant. 2014;49(11):1389-1392.

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SOURCE Jazz Pharmaceuticals plc

Investors: Kathee Littrell, Vice President, Investor Relations, Ireland, +353 1 634 7887, U.S., +1 650 496 2717; Media: Jacqueline Kirby, Vice President, Corporate Affairs & Government Relations, Ireland, +353 1 697 2141, U.S., +1 215 867 4910