Jazz Pharmaceuticals Completes Rolling Submission of New Drug Application for Vyxeos™ (CPX-351), an Investigational Treatment for Acute Myeloid Leukemia

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DUBLIN, April 3, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced the completion on March 31, 2017 of a rolling submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for the approval of Vyxeos™ (cytarabine and daunorubicin) liposome for injection, an investigational treatment for acute myeloid leukemia (AML), a rapidly progressing and life-threatening blood cancer. [1] The company has requested a priority review for the Vyxeos NDA, which, if granted, would accelerate the expected timing of the FDA's review.

Vyxeos utilizes CombiPlex® technology, which encapsulates a fixed ratio of drugs in a nano-scale delivery complex. Vyxeos received Breakthrough Therapy Designation from the FDA in May 2016 for the treatment of adults with therapy-related AML or AML with myelodysplasia-related changes. Vyxeos was also granted Fast Track Designation for the treatment of elderly patients with secondary AML by the FDA, and Orphan Drug Designation by the FDA and the European Commission for the treatment of AML. The FDA grants Breakthrough Therapy Designation to expedite the development and review of new medicines that are intended to treat serious or life-threatening diseases when the clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on at least one clinically significant endpoint.

"The completion of our NDA submission for Vyxeos marks a significant milestone for the company as we continue to advance our pipeline of products for hematology and oncology diseases with high unmet medical needs," said Karen Smith, M.D., Ph.D., global head of research and development and chief medical officer at Jazz Pharmaceuticals. "We look forward to working closely with the FDA toward the goal of obtaining approval of Vyxeos for patients with AML as little has changed in the pharmacologic treatment of AML in more than 40 years."

The NDA submission includes clinical data from five studies, including the pivotal Phase 3 study. Data from the Phase 3 study, which met its primary endpoint, were previously presented at the American Society of Clinical Oncology Annual Meeting in June 2016.

About Vyxeos (CPX-351)

Vyxeos™ (cytarabine and daunorubicin) liposome for injection, or CPX-351, is an investigational product being evaluated for the treatment of AML, and is a combination of 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 FDA and is subject to confirmation upon approval of the NDA.

About Acute Myeloid Leukemia

Acute Myeloid Leukemia (AML) is a rapidly progressing and life-threatening blood cancer that rises in frequency with age.[1] The American Cancer Society estimates that there will be approximately 21,380 new cases of AML and 10,590 deaths from AML in the United States in 2017.[1] The median age at diagnosis is 67 and with rising age there is progressive worsening of prognosis.[1,2] Advancing age is associated with increasing risk of specific chromosomal/mutational changes and risk of pre-malignant marrow disorders, which give rise to more aggressive and less responsive forms of AML.[3,4] As patients age there is also reduced tolerance for intensive chemotherapy.[5] As a consequence, advances in supportive care, intensive chemotherapy and bone marrow transplantation have primarily benefitted younger patients.[3,5] The five-year survival rate has not improved in older patients despite of 40 years of research.[5,6]

About Jazz Pharmaceuticals plc

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 oxbate) 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 1996

This press release contains forward-looking statements, including, but not limited to, statements related to the expected impact of obtaining priority review of the NDA for Vyxeos if such review is granted, working with the FDA toward the goal of approval of the NDA for Vyxeos, the timing of such events and activities, and other statements that are not historical facts. These forward-looking statements are based on the company's current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. 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; the regulatory approval process, including the risks that the company is unable to obtain priority review of the NDA for Vyxeos or obtain FDA approval for Vyxeos in a timely manner or at all; the manufacture and effective commercialization of Vyxeos; 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 period 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] American Cancer Society: Key Statistics About Acute Myeloid Leukemia: *Information accessed on March 20, 2017 from https://www.cancer.org/cancer/acute-myeloid-leukemia/about/key-statistics.html*

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[3] Ferrara F, et al., *Lancet*, 2013 Feb 9- 15 (381):484-495.

[4] Dohner H, et al., *Blood*, 2010; 115(3):453-474.

[5] Stone RM, et al., *Hematology Am Soc Hematol Educ Program*, 2004:98-117.

[6] Kadia TM, *Ann Oncol*. 2016 May 27 (5), 770-8.

SOURCE Jazz Pharmaceuticals plc

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