Jazz Pharmaceuticals Initiates Rolling NDA Submission for Vyxeos (CPX-351) Expects to Complete NDA Submission by Early 2017

October 3, 2016

DUBLIN, Oct. 3, 2016 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq; JAZZ) today announced the initiation of a rolling submission of a New Drug Application (NDA) to the United States (U.S.) Food and Drug Administration (FDA) on September 30, 2016, seeking marketing approval of Vyxeos™ (cytarabine and daunorubicin liposome injection), an investigational agent for the treatment of acute myeloid leukemia (AML). The company expects to complete the submission of the NDA by early 2017, and will request a priority review.

"Our initiation of the rolling NDA submission for Vyxeos brings us closer to an important goal of providing a new treatment option for patients with acute myeloid leukemia, a devastating and life-threatening disease," said Karen Smith M.D., Ph.D., global head of research and development and chief medical officer at Jazz Pharmaceuticals. "AML represents a disease area within the hematological cancers, where there remains a significant unmet medical need. Vyxeos, if approved, would be the first new treatment demonstrating a significant advancement in treating AML in over 20 years."

Vyxeos was granted Breakthrough Therapy Designation in May 2016 for the treatment of adults with therapy-related AML or AML with myelodysplasia-related changes. 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 Breakthrough Therapy designation allows a company to submit individual sections of its NDA for review by the FDA as they are completed rather than waiting until the entire application is complete before it can be submitted and reviewed.

Celator Pharmaceuticals, Inc. completed and announced the results of its Phase 3 trial evaluating Vyxeos in patients with high-risk AML in March 2016. Jazz Pharmaceuticals completed the acquisition of Celator Pharmaceuticals in July 2016.

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 19,950 new cases of AML and 10,430 deaths from AML in the U.S. in 2016.[2] In the European Union, the number of new cases is estimated to be 20,100 in 2016.[3]

The median age at diagnosis is 67 and with rising age there is progressive worsening of prognosis.[1,4] 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.[5,6] As patients age there is also reduced tolerance for intensive chemotherapy.[7] As a consequence, advances in supportive care, intensive chemotherapy, and bone marrow transplantation have primarily benefitted younger patients with approximately one third of patients 18-60 years of age achieving cure.[5,7] Older patients have not achieved higher rates of cure or improved upon a 5-year survival rate of 10-20% in spite of 40 years of research. [7, 8]

About Vyxeos

Vyxeos™ (cytarabine and daunorubicin liposome injection), or CPX-351, is a combination of cytarabine and daunorubicin encapsulated within a nano-scale liposome at a 5:1 molar ratio. Vyxeos was granted orphan drug status by the FDA and the European Commission for the treatment of acute myeloid leukemia. Vyxeos was granted Breakthrough Therapy Designation for the treatment of adults with therapy-related AML or AML with myelodysplasia-related changes and was also granted Fast Track designation by the FDA for the treatment of older patients with secondary AML.

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 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 Jazz Pharmaceuticals' plans to complete a rolling NDA submission for Vyxeos in the U.S. and the timing thereof, and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' 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, the inherent uncertainty associated with the regulatory approval process, including the risk that the company may be unable to obtain or maintain regulatory approval for Vyxeos in the U.S., and other risks and uncertainties affecting the company, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 and future filings and reports by the company. Other risks and uncertainties of which Jazz Pharmaceuticals is not currently aware may also affect its forward-looking statements and may cause actual results and 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 Jazz Pharmaceuticals on its website or otherwise. Jazz Pharmaceuticals does not undertake any obligation to update or supplement any forward-looking statements to reflect actual results, new information, future events, changes in expectations or

other circumstances that exist after the date as of which the forward-looking statements were made.

References:

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SOURCE Jazz Pharmaceuticals plc

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