# Jazz Pharmaceuticals Announces FDA Acceptance of NDA for VYXEOS™ (CPX-351), an Investigational Treatment for Acute Myeloid Leukemia, with Priority Review Status

May 31, 2017

DUBLIN, May 31, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the U.S. Food and Drug Administration (FDA) has accepted for filing with Priority Review its recently submitted New Drug Application (NDA) for VYXEOS™ (cytarabine and daunorubicin) liposome injection, an investigational treatment for acute myeloid leukemia (AML), a rapidly progressing and life-threatening blood cancer.[1]

Priority Review status is designated for drugs that may offer major advances in treatment or provide a treatment where no adequate therapy exists. The granting of Priority Review for the VYXEOS NDA accelerates the timing of the FDA review of the application compared to a standard review.

"We are pleased by the FDA's acceptance of the NDA filing with Priority Review as this action emphasizes the need for new treatments for patients living with AML," said Karen Smith, M.D., Ph.D., executive vice president, research and development and chief medical officer at Jazz Pharmaceuticals. "We look forward to working with the FDA during this review process to obtain approval of VYXEOS as quickly as possible, as AML is the most common of all adult leukemias and AML patients have among the lowest survival rates."[2]

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 presented at the American Society of Clinical Oncology Annual Meeting in June 2016.

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.

**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.[1,3] 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.[4,5] As patients age there is also reduced tolerance for intensive chemotherapy.[6] As a consequence, advances in supportive care, intensive chemotherapy and bone marrow transplantation have primarily benefitted younger patients.[4,6] The five-year survival rate has not improved in older patients despite 40 years of research.[6,7]

**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 timing of and efforts in connection with obtaining FDA approval of the company's NDA for VYXEOS 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; and the regulatory approval process, including the risk that the company is unable to obtain FDA approval for VYXEOS 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 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 April 26, 2017 from https://www.cancer.org*

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