Vyxeos™ Receives Positive CHMP Opinion for Treatment of Certain Types of High-Risk Acute Myeloid Leukaemia

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DUBLIN, June 29, 2018 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) issued a positive opinion recommending marketing authorisation of Vyxeos™ 44 mg/100 mg powder for concentrate for solution for infusion for the treatment of adults with newly diagnosed, therapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC). *Vyxeos* is an advanced liposomal formulation that delivers a fixed-ratio (1:5) of daunorubicin and cytarabine.

"Jazz is committed to bringing new and clinically meaningful treatment options to patients on a global basis, and we now look forward to bringing *Vyxeos* to adults with AML in the European Union," said Allen Yang, M.D., Ph.D., vice president, hematology/oncology therapeutic area head, and acting chief medical officer at Jazz Pharmaceuticals. "If approved by the European Commission, *Vyxeos* will become the first chemotherapy treatment option specifically for European patients with therapy-related AML or AML with myelodysplasia-related changes."

The Marketing Authorisation Application (MAA) for *Vyxeos* 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.

Jazz Pharmaceuticals filed a MAA for *Vyxeos* in November 2017 after the CHMP granted accelerated assessment, which is designed to reduce the review timeline for products of major interest for public health and therapeutic innovation. The positive opinion from the CHMP will be reviewed by the European Commission, which has the authority to approve medicines in all European Union Member States, Iceland, Norway and Liechtenstein.

About Vyxeos™
Vyxeos 44 mg/100 mg powder for concentrate for solution for infusion is an advanced liposomal formulation that delivers a fixed-ratio (1:5) of daunorubicin and cytarabine to the bone marrow that has been shown to have synergistic effects at killing leukaemia cells *in vitro* and in animal models. *Vyxeos* is the first product developed with the company's proprietary CombiPlex® platform, which enables the design and rapid evaluation of various combinations of therapies. *Vyxeos* received Orphan Drug Designation by the European Commission in January 2012 and by the U.S. Food and Drug Administration (FDA) in September 2008 for the treatment of AML. *Vyxeos* received Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare Products Regulatory Agency in the United Kingdom. *Vyxeos* received U.S. FDA approval and orphan drug exclusivity on August 3, 2017 for the treatment of adults with newly-diagnosed t-AML or AML-MRC.

About CombiPlex®
The CombiPlex proprietary technology enables the design and rapid evaluation of various combinations of therapies to deliver enhanced anti-cancer activity. The CombiPlex technology seeks to identify the most synergistic ratio of drugs in vitro and fix this ratio in a nano-scale delivery complex that maintains the synergistic combination after administration. CombiPlex utilizes two proprietary nano-scale delivery platforms: liposomes to control the release and distribution of water-soluble drugs and drugs that are both water- and fat-soluble (amphipathic), and nanoparticles to control the release and distribution of non-water-soluble (hydrophobic) drugs.

About AML
Acute myeloid leukaemia (AML) is a blood cancer that begins in the bone marrow, which produces most of the body's new blood cells.[1] AML cells crowd out healthy cells and move aggressively into the bloodstream to spread cancer to other parts of the body.[2] The median age at diagnosis is 68 years old, with rising age associated with a progressively worsening prognosis.[3-4] There is also a reduced tolerance for intensive chemotherapy as patients age.[5] Patients with t-AML or AML-MRC have few treatment options and some of the lowest survival rates compared to people with other forms of leukaemia.[6-7] A hematopoietic stem cell transplant (HSCT) may be a curative treatment option for patients.[8]

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*), Defitelio® (defibrotide sodium) and Vyxeos™ (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Erwinase® and Defitelio® (defibrotide) in countries outside the U.S. For country-specific product labels, please visit www.jazzpharmaceuticals.com/products. For more information, please visit www.jazzpharmaceuticals.com and follow us on Twitter at @JazzPharma.

"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 potential approval and availability of Vyxeos as a treatment option for European patients with t-AML or AML-MRC 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: the regulatory approval process, including the company's ability to obtain approval for Vyxeos in the EU in a timely manner or at all; the manufacture and effective commercialization of Vyxeos in the EU; 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, 2018 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:

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