Jazz Pharmaceuticals Submits Vyxeos™ Marketing Authorization Application to European Medicines Agency for Treatment of Certain Types of High-Risk Acute Myeloid Leukemia

November 03, 2017

Vyxeos granted accelerated assessment review status from European Medicines Agency's Committee for Medicinal Products for Human Use

DUBLIN, Nov. 3, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) for Vyxeos™ (daunorubicin and cytarabine) powder for concentrate for infusion to treat adults with high-risk acute myeloid leukemia (AML) defined as therapy-related AML (t-AML) or AML with myelodysplasia-related changes (AML-MRC).

The CHMP granted *Vyxeos* accelerated assessment, which is designed to reduce the review timeline for products of major interest for public health and therapeutic innovation. *Vyxeos* also received Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare Products Regulatory Agency in the United Kingdom. A PIM designation is an early indication that a medicinal product is a promising candidate for the Early Access to Medicines Scheme (EAMS), intended for the treatment, diagnosis or prevention of a life-threatening or seriously debilitating condition with the potential to address an unmet medical need.

"If approved, *Vyxeos* will become the first new chemotherapy treatment option specifically for European patients with therapy-related AML or AML with myelodysplasia-related changes," said Karen Smith, M.D., Ph.D., executive vice president, research and development and chief medical officer at Jazz Pharmaceuticals. "We are passionate about bringing a new treatment option for high-risk AML to the appropriate patients in the EU as quickly as possible and look forward to working with the CHMP during this review process."

The MAA for *Vyxeos* includes clinical data from five studies, including the pivotal Phase 3 study. Data from the Phase 3 study, which demonstrated a statistically significant improvement in overall survival for *Vyxeos* versus standard of care, were presented at the American Society of Clinical Oncology Annual Meeting in June 2016.

"Despite numerous advances in the treatment of cancer in Europe, patients with high-risk AML defined as t-AML or AML-MRC have limited treatment options and some of the lowest survival rates compared to people with other forms of AML," said Dr. Nigel Russell, Professor of Haematology, Faculty of Medicine & Health Sciences at the University of Nottingham. "The hematology oncology community has worked tirelessly to better understand the causes and disease pathways associated with AML and we look forward to the arrival of innovative new treatment options."

About Vyxeos ™

Vyxeos daunorubicin 2.2 mg/mL and cytarabine 5 mg/mL powder for concentrate 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 leukemia 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 U.S. FDA approval on August 3, 2017 for the treatment of adults with newly-diagnosed t-AML or AML-MRC.

About AML

Acute myeloid leukemia (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 leukemia.[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.jazzpharma.com/products. 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 Vyxeos as a treatment option for European patients with t-AML or AML-MRC, the expected impact of accelerated assessment review status on the Vyxeos MAA, 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 maintain accelerated assessment review status for the Vyxeos MAA or obtain approval for Vyxeos in the EU in a timely manner or at all; and 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 June 30, 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. National Cancer Institute. General Information About Adult Acute Myeloid Leukemia https://www.cancer.gov/types/leukemia/patient/adult-aml-treatment-pdq Accessed June 7, 2017.
2. American Cancer Society. What is Acute Myeloid Leukemia? https://www.cancer.org/cancer/acute-myeloid-leukemia/about/what-is-aml.html Accessed March 20, 2017.
3. SEER Stat Facts: AML. 2017.
4. Baer MR, et al., Leukemia, 2011 May; 25(5):10.1038/eu.2011.9.
5. Klepin HD. Hematology Am Soc Hematol Educ Program. 2014 (1):8-13.
6. Pinto AC, et al. Current Cancer Treatment - Novel Beyond Conventional Approaches. 2011; 693-714.
7. Smith SM, et al. *Blood*. 2003; 102(1):43-52.
8. Peccatori, J and Ciceri, F. Haematologica. 2010 Jun; 95(6): 857–859. doi: 10.3324/haematol.2010.023184.

View original content with multimedia:http://www.prnewswire.com/news-releases/jazz-pharmaceuticals-submits-vyxeos-marketing-authorization-application-to-european-medicines-agency-for-treatment-of-certain-types-of-high-risk-acute-myeloid-leukemia-300549237.html

SOURCE Jazz Pharmaceuticals plc

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