National Institute for Health and Care Excellence (NICE) Recommends Jazz Pharmaceuticals' Vyxeos® (Daunorubicin and Cytarabine) for Adults with Specific Types of Secondary Acute Myeloid Leukaemia (AML)

November 8, 2018

DUBLIN, Nov. 7, 2018 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the National Institute for Health and Care Excellence (NICE) has published a Final Appraisal Determination (FAD) recommending Vyxeos® 44 mg/100 mg powder for concentrate for solution for infusion for routine use on the National Health Service (NHS) in England and Wales for the treatment of adults with newly diagnosed, therapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC)[1] – two types of secondary AML.

"This is the first new chemotherapy in forty years for adults with specific types of newly diagnosed secondary AML, a particularly aggressive cancer that typically affects older people and has a high mortality rate," said Dr. Nigel Russell, Professor of Haematology, Faculty of Medicine & Health Sciences at the University of Nottingham. "I am pleased that NICE has recognised the value of this medicine for adults with secondary AML. In time, it is expected to become the standard of care for this specific group of older AML patients."

Patients diagnosed with t-AML or AML-MRC have a very poor prognosis and have the poorest survival of all AML diagnostic subgroups.[2,3] Thus they are classified as having high risk disease because of these poor outcomes. In the UK, the expected number of these high-risk AML cases is 680 per year.[4] Its incidence increases with age and accounts for approximately 25% of all AML cases in the UK.[4]

"A diagnosis of acute myeloid leukaemia can have a huge emotional impact on the lives of patients, as well as their family and friends," said Zack Pemberton-Whiteley, Patient Advocacy Director at Leukaemia Care. "We welcome the decision to recommend this treatment for adults with high-risk AML. Leukaemia Care has been working with NICE to enable patients to access this important new medicine, where options have been previously limited."

"Jazz is delighted that *Vyxeos* will now be made routinely available on the NHS in England and Wales as people with therapy-related AML or AML with myelodysplasia-related changes have had limited treatment options until now," said Iain McGill, senior vice president, Europe and Rest of World at Jazz Pharmaceuticals. "We believe that it is a meaningful medicine for patients with this rapidly progressing and life-threatening blood cancer."

Vyxeos is an advanced liposomal formulation that delivers a synergistic molar ratio of daunorubicin and cytarabine. It is the first chemotherapy to demonstrate a significant overall survival advantage versus the current treatment standard, 7+3 chemotherapy, in a Phase 3 study of older adult patients with newly diagnosed t-AML or AML-MRC,[5] and the first chemotherapy treatment option specifically for people who have these types of high-risk AML.

Notes to Editors

About Vyxeos®
It is an advanced liposomal formulation that has been shown in vitro to deliver a synergistic combination of daunorubicin and cytarabine to leukaemia cells for a prolonged period of time. Based on data in animals, the liposomes accumulate and persist in high concentration in the bone marrow, where they are preferentially taken up intact by leukaemia cells.[6] It is the first product developed with the company's proprietary CombiPlex® platform, which enables the consideration and design of various combinations of therapies.

It received Orphan Drug Designation (ODD) by the European Commission in January 2012 with retention of the ODD reaffirmed in July 2018 following assessment by the Committee for Orphan Medicinal Products (COMP), and by the U.S. Food and Drug Administration (FDA) in September 2008 for the treatment of AML. It received Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare Products Regulatory Agency in the United Kingdom. In August 2018, the European Commission approved it for the treatment of adults with newly diagnosed t-AML or AML-MRC in all European Union Member States, as well as Iceland, Norway and Liechtenstein.

View the Summary of Product Characteristics here.

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. AML cells crowd out healthy cells and move into the bloodstream to spread cancer to other parts of the body.[7] The median age at diagnosis is 72 years old, with rising age associated with a progressively worsening prognosis.[4] There is also a reduced tolerance for intensive chemotherapy as patients age.[8] Patients with t-AML or AML-MRC have the lowest survival rates compared with people with other forms of leukaemia.[2,3] A haematopoietic stem cell transplant (HSCT) may be a curative treatment option for patients.[9]

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.

"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* becoming the standard of care for patients with newly diagnosed 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 company's ability to effectively commercialize *Vyxeos*; delays or problems in the supply or manufacture of *Vyxeos*; maintaining appropriate pricing and reimbursement for *Vyxeos*; complying with applicable regulatory requirements; 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 September 30, 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.

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