CMS Grants New Technology Add-On Payment to Vyxeos® (daunorubicin and cytarabine) Liposome for Injection

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DUBLIN, Aug. 3, 2018 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the United States Centers for Medicare and Medicaid Services (CMS) granted approval for a New Technology Add-on Payment (NTAP) for Vyxeos® (daunorubicin and cytarabine) liposome for injection for the treatment of adults with newly diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC), a rapidly progressing and life-threatening blood cancer.

The *Vyxeos* NTAP will support Medicare beneficiaries' access to *Vyxeos* when they are treated in certain inpatient hospital settings. The NTAP payment is in addition to the diagnosis-related group (MS-DRG)-based reimbursement that hospitals receive under the Medicare Hospital Inpatient Prospective Payment System (IPPS). The NTAP designation is expected to remain in effect for a period of two to three years until the cost of *Vyxeos* is included in CMS's recalibration of the DRG payment rate.

The NTAP program is only available to new technologies meeting the definition of newness of the technology, exceeding cost criterion thresholds and demonstrating substantial clinical improvement over existing therapies.

"We value the decision by CMS to grant NTAP designation for *Vyxeos* as it underscores our belief that *Vyxeos* is an important treatment option for patients with newly-diagnosed t-AML or AML-MRC," said Mike Miller, executive vice president and U.S. commercial lead at Jazz Pharmaceuticals. "AML is a blood cancer most often seen in older adults and the NTAP payment will help improve Medicare beneficiaries' access to *Vyxeos*, the first FDA-approved treatment specifically for these patients."

Vyxeos was approved by the U.S. Food and Drug Administration in August 2017 and is the second product from Jazz Pharmaceuticals to receive a NTAP.

***Vyxeos* has different dosage recommendations from other medications that contain daunorubicin and/or cytarabine. Do not substitute *Vyxeos* for other daunorubicin- and/or cytarabine- containing products.**

In the final rule concerning Hospital IPPS and Fiscal Year 2019, (scheduled for publication in the Federal Register on August 17, 2018), CMS states that: "After consideration of the public comments we received, we believe that based on the statistically significant increase in median survival rate from the Phase III Study 301, *Vyxeos* is a treatment option which offers a substantial clinical improvement over standard therapy for patients who have been diagnosed with AML. Therefore, we believe that *Vyxeos* meets the substantial clinical improvement criterion. Based on evaluation of the new technology add-on payment application and consideration of the public comments we received, we have determined that *Vyxeos* meets all of the criteria for approval for new technology add-on payments. Therefore, we are approving new technology add-on payments for *Vyxeos* for FY 2019."

Effective October 1, 2018, CMS has established the maximum NTAP amount of $36,425.00, which will provide incremental reimbursement of up to 50 percent of the *Vyxeos* Wholesale Acquisition Cost (WAC) of an average maximum of 9.4 vials when used in the IPPS hospital setting. Additional information on the CMS final rule and its discussion of NTAP and *Vyxeos* can be found on pages 551-575 of the pre-publication Federal Register: https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/AcuteInpatientPPS/FY2019-IPPS-Final-Rule-Home-Page-Items/FY2019-IPPS-Final-Rule-Regulations.html?DLPage=1&DLEntries=10&DLSort=0&DLSortDir=ascending

Introduced in 2001, NTAP was created by Congress to help facilitate access to new, innovative technologies used to treat Medicare beneficiaries in the hospital inpatient setting.

About Vyxeos®
Vyxeos® (daunorubicin and cytarabine) liposome for injection 44mg/100mg is a liposome formulation of a fixed combination of daunorubicin and cytarabine for intravenous infusion.[1] *Vyxeos* is indicated for the treatment of adults with newly-diagnosed t-AML or AML-MRC. For more information about *Vyxeos* in the United States, please visit https://vyxeos.com.

Important Safety Information

Vyxeos has different dosage recommendations from other medications that contain daunorubicin and/or cytarabine. Do not substitute Vyxeos for other daunorubicin- and/or cytarabine- containing products.

Vyxeos should not be given to patients who have a history of serious allergic reaction to daunorubicin, cytarabine or any of its ingredients.

Vyxeos can cause a severe decrease in blood cells (red and white blood cells and cells that prevent bleeding, called platelets) which can result in serious infection or bleeding and possibly lead to death. Your doctor will monitor your blood counts during treatment with Vyxeos. Patients should tell the doctor about new onset fever or symptoms of infection or if they notice signs of bruising or bleeding.

Vyxeos can cause heart-related side effects. Tell your doctor about any history of heart disease, radiation to the chest, or previous chemotherapy. Inform your doctor if you develop symptoms of heart failure such as:

- shortness of breath or trouble breathing
- swelling or fluid retention, especially in the feet, ankles or legs
- unusual tiredness

Vyxeos may cause allergic reactions including anaphylaxis. Seek immediate medical attention if you develop signs and symptoms of anaphylaxis such

as:

- trouble breathing
- severe itching
- skin rash or hives
- swelling of the face, lips, mouth, or tongue

Vyxeos contains copper and may cause copper overload in patients with Wilson's disease or other copper-processing disorders.

Vyxeos can damage the skin if it leaks out of the vein. Tell your doctor right away if you experience symptoms of burning, stinging, or blisters and skin sores at the injection site.

Vyxeos can harm your unborn baby. Inform your doctor if you are pregnant, planning to become pregnant, or nursing. Do not breastfeed while receiving Vyxeos. Females and males of reproductive potential should use effective contraception during treatment and for 6 months following the last dose of Vyxeos.

The most common side effects were bleeding events, fever, rash, swelling, nausea, sores in the mouth or throat, diarrhea, constipation, muscle pain, tiredness, stomach pain, difficulty breathing, headache, cough, decreased appetite, irregular heartbeat, pneumonia, blood infection, chills, sleep disorders, and vomiting.

Please see full Prescribing Information for Vyxeos including BOXED Warning at: http://pp.jazzpharma.com/pi/vyxeos.en.USPI.pdf

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. AML cells crowd out healthy cells and move aggressively into the bloodstream to spread cancer to other parts of the body.[2] AML is a relatively rare disease representing 1.1 percent of all new cancer cases.[3] It is estimated that approximately 19,500 people will be diagnosed with AML in the United States this year with the potential for nearly 11,000 people to die from the disease.[4] The median age at diagnosis is 68 years old, with rising age associated with a progressively worsening prognosis.[3,5] There is also a reduced tolerance for intensive chemotherapy as patients age.[6] Patients with newly diagnosed t-AML or AML-MRC may have a particularly poor prognosis.[7-9]A hematopoietic stem cell transplant (HSCT) may be a curative treatment option for patients.[10]

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 Erwinaze® and Defitelio® (defibrotide) in countries outside the U.S. For country - specific product information more information, please visit www.jazzpharmaceuticals.com/products. For more information, please visit www.jazzpharmaceuticals.com and follow us on Twitter @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 expected duration and impact of the NTAP designation 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 the extent to which hospitals will take advantage of the NTAP program with respect to Vyxeos 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.

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