Jazz Pharmaceuticals to Highlight Hematology Research at ASH 2018 Annual Meeting

November 28, 2018

Nine abstracts, including two oral presentations, accepted for Jazz hematology/oncology portfolio, focusing on leukemia and complications of stem cell transplantation

DUBLIN, Nov. 28, 2018 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that nine abstracts, including two oral presentations, relating to the company's hematology/oncology portfolio were accepted for the 60th American Society of Hematology (ASH) Annual Meeting in San Diego from December 1-4.

"We look forward to showcasing our commitment to the hematology/oncology community at ASH by advancing the science and addressing the clinical needs of patients with blood cancers and with complications of stem cell transplantation," said Allen Yang, M.D., Ph.D., head of clinical development and acting chief medical officer of Jazz Pharmaceuticals. "We've made great progress in the last year with the initiation of new clinical trials for *Vyxeos* and *Defitelio*, the marketing authorization of *Vyxeos* in the European Union, and a collaboration with MD Anderson Cancer Center to evaluate potential treatment options for hematologic malignancies."

Jazz Pharmaceuticals data at the 2018 ASH Annual Meeting will highlight the following:

Results from a post-hoc analysis from the pivotal Phase 3 randomized trial of Vyxeos® (daunorubicin and cytarabine) liposome for injection, also known as the molecule name CPX-351, in a subgroup of older patients with newly diagnosed high risk (secondary) AML with myelodysplasia-related changes (AML-MRC) that evaluated the efficacy of *Vyxeos* compared to conventional 7+3 chemotherapy on overall survival and remission rates as well as the safety profile in older adults with AML-MRC. Additionally, data will be presented from an exploratory analysis which evaluated the impact of hematopoietic cell transplantation (HCT) on survival in patients treated with *Vyxeos* compared to 7+3 in the Phase 3 trial.

Vyxeos abstracts include:

- Efficacy and Safety of CPX-351 versus 7+3 in a Subgroup of Older Patients with Newly Diagnosed Acute Myeloid Leukemia with Myelodysplasia-Related Changes (AML-MRC) Enrolled in a Phase 3 Study [Abstract #1425; Saturday, December 1, 6:15 PM – 8:15 PM PST]
- Population Pharmacokinetic (PK)/Pharmacodynamic (PD) Modeling of Myelosuppression in Patients with Hematologic Malignancies for CPX-351 and Standard-of-Care 7+3 Therapy [Abstract #4037; Monday, December 3, 6:00 PM – 8:00 PM PST]
- The Impact of Hematopoietic Cell Transplantation on Survival: An Exploratory Analysis of a Phase 3 Study of CPX-351 versus 7+3 in Older Patients with Newly Diagnosed, High-Risk/Secondary AML [Abstract #2706; Sunday, December 2, 6:00 PM – 8:00 PM PST]
- Final Safety and Efficacy Results from the CPX-351 Early Access Program (EAP) for Older Patients with High-Risk/Secondary Acute Myeloid Leukemia (sAML) [Abstract #1434; Saturday, December 1, 6:15 PM – 8:15 PM PST]
- Impact of Post–Hematopoietic Cell Transplant (HCT) Survival on Cost-effectiveness of CPX-351 versus 7+3 in the Treatment of Therapy-Related AML or AML-MRC in the United States (online only)
- A Phase I/Pilot Study of CPX-351 [Daunorubicin and Cytarabine Liposome for Injection (Vyxeos®)] for Children, Adolescents and Young Adults with Recurrent or Refractory Acute Leukemia [Abstract #336; Sunday, December 2, 10:45 AM PST(oral presentation)]

In addition, data from an expanded access program and post-hoc analyses of clinical trials for Defitelio® (defibrotide sodium) will be presented, including an oral presentation of a pooled analysis of survival based on timing of initiation in adults with Veno-Occlusive Disease/ Sinusoidal Obstruction Syndrome (VOC/SOS) following hematopoietic stem cell transplant (HSCT).

Defitelio abstracts include:

- Incidence of Post-Hematopoietic Stem Cell Transplantation (HSCT) Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) Without Hyperbilirubinemia at Diagnosis and Efficacy of Defibrotide in an Expanded-Access Program [Abstract #2080; Saturday, December 1, 6:15 PM – 8:15 PM PST]
- A Pooled Analysis of Survival by Defibrotide Timing of Initiation in Adults with Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) Following Hematopoietic Stem Cell Transplant (HSCT) [Abstract #815; Monday, December 3, 3:45 PM PST (oral presentation)]
- Cost-Effectiveness of Defibrotide for the Treatment of Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) with Multi-Organ Dysfunction (MOD) Post-Hematopoietic Stem Cell Transplantation (HSCT) in Canada [Abstract #4702; Monday, December 3, 6:00 PM – 8:00 PM PST]

About Vyxeos®

Vyxeos® (daunorubicin and cytarabine) is a liposome formulation of a fixed combination of daunorubicin and cytarabine for intravenous infusion, and is approved for the treatment of two types of secondary AML in adult patients, newly diagnosed therapy-related acute myeloid leukaemia (t-AML) and

AML with myelodysplasia-related changes (AML-MRC). *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 U.S. FDA approval and orphan drug exclusivity in August 2017 and EU EMA marketing authorization in August 2018. *Vyxeos* received Orphan Drug Designation for the treatment of AML by the U.S. FDA in September 2008 and by the European Commission in January 2012 (with retention of the designation reaffirmed in July 2018). *Vyxeos* received Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare Products Regulatory Agency in the United Kingdom.

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, and visit www.Vyxeos.com for additional information.

About Defitelio®
In the U.S., Defitelio® (defibrotide sodium) injection 80mg/mL received U.S. FDA marketing approval on March 30, 2016 for the treatment of adult and pediatric patients with hepatic veno-occlusive disease (VOD), also known as sinusoidal obstruction syndrome (SOS), with renal or pulmonary dysfunction following hematopoietic stem-cell transplantation (HSCT) and is the first and only FDA-approved therapy for patients with this rare, potentially fatal complication.

In Europe, defibrotide is marketed under the name Defitelio®* (defibrotide). In October 2013, the European Commission granted marketing authorization to Defitelio under exceptional circumstances for the treatment of severe VOD in patients undergoing HSCT therapy. It is the first and only approved treatment in Europe for severe VOD. In Europe, Defitelio is indicated in patients over one month of age. It is not indicated in patients with hypersensitivity to defibrotide or any of its excipients or with concomitant use of thrombolytic therapy.

*This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system found under section 4.8 of the SmPC. (https://www.ema.europa.eu/documents/product-information/defitelio-epar-product-information_en.pdf)

Important Safety Information

Defitelio should not be given to patients who are:

- Currently taking anticoagulants or fibrinolytics
- Allergic to Defitelio or any of its ingredients

Defitelio may increase the risk of bleeding in patients with VOD and should not be given to patients with active bleeding. During treatment with Defitelio, patients should be monitored for signs of bleeding. In the event that bleeding occurs during treatment with Defitelio, treatment should be temporarily or permanently stopped. Patients should tell the doctor right away about any signs or symptoms of hemorrhage such as unusual bleeding, easy bruising, blood in urine or stool, headache, confusion, slurred speech, or altered vision.

Defitelio may cause allergic reactions including anaphylaxis. Patients who develop signs and symptoms of anaphylaxis such as trouble breathing, severe itching, skin rash or hives, or swelling of the face, lips, mouth or tongue should seek medical attention immediately.

The most common side effects of Defitelio are decreased blood pressure, diarrhea, vomiting, nausea and nose bleeds.

Please see full Prescribing Information for Defitelio and visit www.Defitelio.com for additional information.

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 therapeutic 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®, Defitelio® (defibrotide) and Vyxeos® 44 mg/100 mg powder for concentrate for solution for infusion in countries outside the U.S. For country-specific product information, please visit http://www.jazzpharmaceuticals.com/products. For more information, please visit http://www.jazzpharmaceuticals.com/ and follow us on Twitter at @JazzPharma.

View original content to download multimedia:http://www.prnewswire.com/news-releases/jazz-pharmaceuticals-to-highlight-hematology-research-at-ash-2018-annual-meeting-300756774.html

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