Jazz Pharmaceuticals to Present New Data at Upcoming ASCO Annual Meeting and EHA Congress

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Data to be presented underscore commitment to developing life-changing medicines in hematology/oncology for people with limited or no treatment options

DUBLIN, May 16, 2019 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that it will present data from across its hematology/oncology portfolio and pipeline at the upcoming American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago from May 31-June 4, 2019 and at the 24thCongress of the European Hematology Association (EHA) in Amsterdam from June 13-16, 2019.

"We are excited about our data presentations at both ASCO and EHA as they demonstrate our growing commitment to research in hematology and oncology, and we are also looking forward to data to be presented by the Children's Oncology Group at ASCO from its Phase 1/2 study of liposomal daunorubicin and cytarabine (CPX-351) for children with relapsed acute myeloid leukemia (AML)," said Allen Yang, M.D., Ph.D., senior vice president of clinical development and acting chief medical officer of Jazz Pharmaceuticals. "Jazz is committed to changing the lives of people with limited or no treatment options who are suffering from blood cancers or complications of stem cell transplantation, and we are hopeful our data at both ASCO and EHA will get us one step closer to that goal for the patients who can benefit from our medicines."

"These are exciting results for children with relapsed AML. We plan to study the use of CPX-351 further in newly diagnosed pediatric AML patients through a Children's Oncology Group (COG) Phase 3 study in partnership with Jazz and the National Cancer Institute," said Todd Cooper, D.O., professor of Pediatrics at the University of Washington, School of Medicine; director of the Pediatric Leukemia/Lymphoma Program at Seattle Children's Cancer and Blood Disorders Center, Evans Family Endowed Chair of Pediatric Cancer and chairman of COG's Acute Myeloid Leukemia New Agents Committee.

Highlights at ASCO will include:

- Oral presentation from the Children's Oncology Group examining CPX-351 (liposomal daunorubicin and cytarabine), also known as Vyxeos®, followed by fludarabine, cytarabine, and G-CSF (FLAG) for children with relapsed AML.
- Poster presentation summarizing outcomes with CPX-351 versus 7+3 by baseline bone marrow blast percentage in older adults with newly diagnosed, high-risk/secondary AML.

Highlights at EHA will include:

- Poster presentation summarizing the interim results from a real-world evidence study, DEFIFrance, of Defitelio® (defibrotide) treatment in adults with severe or very severe veno-occlusive disease/sinusoidal obstruction syndrome after hematopoietic cell transplantation.
- Poster presentation analyzing the outcomes with CPX-351 versus 7+3 in patients who achieved remission.

The Jazz-supported poster presentation and the partner oral presentation covering CPX-351 at the ASCO Annual Meeting are:

Children's Oncology Group Oral Presentation

Presentation Title	Author	Date / Time / Session/ Presentation Number/ Location
AAML 1421, A Phase 1/2 Study of CPX-351 Followed by Fludarabine, Cytarabine, and G-CSF (FLAG) for Children with Relapsed Acute Myeloid Leukemia (AML): A Report from the Children's Oncology Group	Cooper et al.	Friday, May 31, 2019 / 3:45 – 3:57 p.m. CDT Session: Pediatric Oncology I Number: 10003 Location: S504

CPX-351 Poster Presentation

Presentation Title	Author	Date / Time / Session/ Presentation Number/ Location
Outcomes With CPX-351 Versus 7+3 by Baseline Bone Marrow Blast Percentage in Older Adults With Newly Diagnosed, High-risk/Secondary AML: Exploratory Analysis of a Phase 3 Study	Ritchie et al.	Monday, June 3, 2019 / 8:00 – 11:00 a.m. CDT Number: 417 Location: Hall A

The Jazz-supported poster presentations covering defibrotide and CPX-351 at the EHA 24thCongress are:

Defibrotide Poster Presentation

Presentation Title	Author	Date / Time / Session/ Presentation Number/ Location
Defibrotide Treatment in Adults With Severe or Very Severe Veno-occlusive Disease/Sinusoidal Obstruction Syndrome After Hematopoietic Cell Transplantation: DEFIFrance Study Interim Results	Yakoub-Agha et al.	Friday June 14, 5:30 – 7:00 p.m. CET Session: Stem Cell Transplantation – Clinical Number: PF747 Location: Poster area

CPX-351 Poster Presentation

Presentation Title	Author	Date / Time / Session/ Presentation Number/ Location

Phase 3 Exploratory Analysis of Outcomes in Older Adults With Newly Diagnosed, High-risk/Secondary AML Who Achieved Remission With CPX-351 Versus 7+3 Induction	Faderl et al.	Friday June 14, 5:30 – 7:00 p.m. CET Session: Acute Myeloid Leukemia – Clinical Number: PF293 Location: Poster area

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. Food and Drug Administration (FDA) approval and orphan drug exclusivity in August 2017 and EU European Medicines Agency (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 for Vyxeos

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).

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.

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 for Defitelio

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), a global biopharmaceutical company, is dedicated to developing life-changing medicines for people with limited or no options. As a leader in sleep medicine and with a growing hematology/oncology portfolio, Jazz has a diverse portfolio of products and product candidates in development, and is focused on transforming biopharmaceutical discoveries into novel medicines. Jazz Pharmaceuticals markets Xyrem® (sodium oxybate) oral solution, Defitelio® (defibrotide sodium), Erwinaze® (asparaginase *Erwinia chrysanthemi*), and Vyxeos® (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Defitelio® (defibrotide), Erwinase®, 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 https://www.jazzpharma.com/medicines. For more information, please visit www.jazzpharmaceuticals.com and follow us on Twitter at @JazzPharma.

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