Jazz Pharmaceuticals to Highlight Growing Hematology Oncology Pipeline at ASH 2019 Annual Meeting

November 07, 2019

15 abstracts spanning Jazz's hematology/oncology therapeutic area focusing on acute leukemias and complications of stem cell transplantation

New sub-analysis of Vyxeos Phase 3 trial data accepted as an oral presentation

DUBLIN, Nov. 7, 2019 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that 15 abstracts sponsored by Jazz Pharmaceuticals, including one oral presentation, one abstract from a collaboration trial with MD Anderson Cancer Center (MD Anderson) and two abstracts from investigator-sponsored trials, will be presented at the 61st American Society of Hematology (ASH) Annual Meeting in Orlando from December 7-10, 2019.

"Jazz's ASH presence reflects our commitment to developing new medicines for patients who have limited or no treatment options," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development of Jazz Pharmaceuticals. "We are pleased with the progress we made over the last year to initiate new clinical trials and cooperative group studies, and further expanding our oncology pipeline."

Highlights at ASH will include:

- An oral presentation comparing outcomes for Vyxeos® (daunorubicin and cytarabine), also known as CPX-351, versus 7+3 in the Study 301 subgroup of patients with pretreatment blood or bone marrow samples available evaluating secondary acute myeloid leukemia (AML) patient genetic characteristics and the association between gene mutations and outcomes
- A poster presentation comparing outcomes for CPX-351 versus 7+3 in the Study 301 subgroup of patients who met the World Health Organization 2008 AML with myelodysplasia-related changes criteria and achieved a complete remission (CR) or CR with incomplete neutrophil or platelet recovery (CRi)
- A poster presentation comparing outcomes for CPX-351 versus 7+3 in the Study 301 subgroup of patients with therapy-related AML who achieved a CR or CRi
- A poster presentation from a collaboration study with MD Anderson evaluating CPX-351 in combination with gemtuzumab ozogamicin in relapsed refractory patients with AML and post-hypomethylating agent failure high-risk myelodysplastic syndrome
- A poster presentation summarizing safety and outcome data from patients with severe veno-occlusive disease/sinusoidal obstruction syndrome after hematopoietic cell transplantation who were treated with defibrotide and assessing patterns of defibrotide utilization in the post-approval setting

A full list of Jazz-sponsored oral and poster presentations follows below:

Vyxeos (CPX-351) Presentations

Presentation Title	Author	Date / Time (ET) / Session / Location
Genetic Characteristics and Outcomes by Mutation Status in a Phase 3 Study of CPX-351 Versus 7+3 in Older Adults with Newly Diagnosed, High-Risk/Secondary Acute Myeloid Leukemia (AML)	Lindsley et al.	Oral presentation: Saturday, December 7 8:00 a.m., Room W304 613. Acute Myeloid Leukemia: Clinical Studies: Prognostic Factors and Treatment Outcomes
Efficacy and Safety of CPX-351 Versus 7+3 in a Phase 3 Exploratory Analysis in Patients with High-Risk/Secondary Acute Myeloid Leukemia (AML) with Prior Hypomethylating Agent Exposure Who Achieved Remission	Lin et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 613. Acute Myeloid Leukemia: Clinical Studies: Poster I
A Descriptive Study on Healthcare Utilization and Costs in Secondary Acute Myeloid Leukemia (AML) Patients Treated with CPX-351 Versus Those Treated with 7+3	Price et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 906. Outcomes Research—Malignant Conditions (Myeloid Disease): Poster I

Quantifying the Economic Burden of Myelodysplastic Syndromes Among Elderly US Patients	Shafrin et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 903. Health Services Research —Malignant Conditions (Myeloid Disease): Poster I
Outcomes in Patients with Therapy-Related Acute Myeloid Leukemia (t-AML) Who Achieved Remission with CPX-351 Versus 7+3: Phase 3 Exploratory Analysis	Lancet et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 613. Acute Myeloid Leukemia: Clinical Studies: Poster I
Outcomes in Patients with Acute Myeloid Leukemia with Myelodysplasia-Related Changes (AML-MRC) Who Achieved Remission with CPX-351 Versus 7+3: Phase 3 Exploratory Analysis	Ryan et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 613. Acute Myeloid Leukemia: Clinical Studies: Poster III
Phase 3 Exploratory Analysis of Outcomes in Patients with Acute Myeloid Leukemia with Myelodysplasia-Related Changes (AML-MRC) Who Received Consolidation with CPX-351 Versus Conventional Chemotherapy	Kolitz et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 613. Acute Myeloid Leukemia: Clinical Studies: Poster III

Defibrotide Presentations

Presentation Title	**Author**	**Date / Time (ET) / Session / Location**
A Multi-Center, Multinational, Prospective Observational Registry Study of Defibrotide in Patients Diagnosed with Severe Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) after Hematopoietic Cell Transplantation (HCT)	Mohty et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster I
A Meta-Analysis Evaluating the Risk of Bleeding-Related Adverse Events with Defibrotide Treatment	Tappe et al.	Saturday, December 7 5:30 p.m. – 7:30 p.m., Hall B 723. Clinical Allogeneic and Autologous Transplantation: Late Complications and Approaches to Disease Recurrence: Poster I
Incidence and Cost of Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome with and without Multi-Organ Dysfunction: Analysis of the Premier Healthcare Database	Dvorak et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 901. Health Services Research—Non-Malignant Conditions: Poster III

JZP-458 Presentations

Presentation Title	**Author**	**Date / Time (ET) / Session / Location**
Open-label, Multicenter, Phase 2/3 Study of Recombinant Crisantaspase Produced in *Pseudomonas fluorescens* in Patients with Acute Lymphoblastic Leukemia (ALL) or Lymphoblastic Lymphoma (LBL) Following Hypersensitivity to *Escherichia coli*–derived Asparaginases	Maese et al.	Sunday, December 8 6:00 p.m. – 8:00 p.m., Hall B 612. Acute Lymphoblastic Leukemia: Clinical Studies: Poster II
A Phase 1 Study of the Safety, Tolerability, and Pharmacokinetics of Recombinant Crisantaspase Produced in *Pseudomonas fluorescens* (RC-P) in Healthy Adults	Hernandez-Illas et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 612. Acute Lymphoblastic Leukemia: Clinical Studies: Poster III

Additionally, data from the following investigator-sponsored and collaboration trials on Vyxeos and Defitelio, respectively, will be presented:

Presentation Title	**Author**	**Date / Time (ET) / Session / Location**
Liposomal Cytarabine and Daunorubicin (CPX-351) in Combination with Gemtuzumab Ozogamicin (GO) in Relapsed Refractory (R /R) Patients with Acute Myeloid Leukemia (AML) and Post-Hypomethylating Agent (Post-HMA) Failure High-Risk Myelodysplastic Syndrome (HR-MDS) [MD Anderson collaboration trial]	Ramos Perez et al.	Sunday, December 8 6:00 p.m. – 8:00 p.m., Hall B 615. Acute Myeloid Leukemia:

		Commercially Available Therapy, excluding Transplantation: Poster II
CPX-351 As First Intensive Therapy for Elderly Patients with AML	Ritchie et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 613. Acute Myeloid Leukemia: Clinical Studies: Poster III
Defibrotide Inhibits Endothelial Cell Injury Induced by Plasmas of Patients with Thrombotic Microangiopathies	Elhadad et al.	Monday, December 9 6:00 p.m. – 8:00 p.m., Hall B 332. Anticoagulation and Antithrombotic Therapy: Poster III

About Vyxeos® (daunorubicin and cytarabine)

Vyxeos® (daunorubicin and cytarabine) is a liposomal formulation of a fixed combination of daunorubicin and cytarabine for intravenous infusion that represents the first, only and most proven chemotherapy treatment option specifically for two types of high-risk, secondary AML: newly diagnosed therapy-related acute myeloid leukemia (AML) and AML with myelodysplasia-related changes. Backed by a robust clinical development program including Phase 3 data, Vyxeos is currently approved in more than 30 countries including the U.S., and we continue to work with regulatory authorities worldwide to bring this innovative therapy to appropriate patients.

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® (defibrotide sodium)

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, 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**.

(http://www.ema.europa.eu/ema/index.jsp?curl=/pages/medicines/human/medicines/002393/human_med_001646.jsp)

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 JZP-458
JZP-458 is a recombinant *Erwinia* asparaginase that uses a novel *Pseudomonas fluorescens* expression platform. It is being developed for use as a component of a multi-agent chemotherapeutic regimen in the treatment of pediatric and adult patients with acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL) who are hypersensitive to *E. coli*-derived asparaginase products. JZP-458 was granted Fast Track designation by the U.S. Food and Drug Administration in October 2019 for the treatment of this patient population.

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 Sunosi® (solriamfetol), Xyrem® (sodium oxybate) oral solution, Defitelio® (defibrotide sodium), Erwinaze® (*Erwinia asparaginase*) and Vyxeos® (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Xyrem®, Defitelio® (defibrotide), Erwinase® and Vyxeos® liposomal 44 mg/100 mg powder for concentrate for solution for infusion in countries outside the U.S. For country-specific product information, please visit www.jazzpharmaceuticals.com/medicines. For more information, please visit www.jazzpharmaceuticals.com and follow us on Twitter at @JazzPharma.

Media Contact:
Jacqueline Kirby, Vice President, Corporate Affairs & Government Relations Ireland +353 1 697 2141 U.S. +1 215 867 4910

Investor Contact:
Kathee Littrell, Vice President, Investor Relations Ireland +353 1 634 7887 U.S. +1 650 496 2717

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