Jazz Pharmaceuticals to Showcase New Data From its Growing Oncology Portfolio at Virtual ASCO and EHA 2020 Meetings

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Findings advance research across multiple therapeutic areas for adult and pediatric patients with rare or complex diseases

DUBLIN, May 14, 2020 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that it and its partners will present seven new abstracts at the virtual American Society of Clinical Oncology (ASCO) Annual Meeting from May 29-June 2, 2020 and six abstracts at the virtual 25th Annual Congress of the European Hematology Association (EHA) from June 11-14, 2020. These new data from Jazz's expanding hematology and oncology portfolio and pipeline are focused on difficult-to-treat cancers, or related disorders, which have few, if any, treatment options.

"Jazz is committed to oncology, including developing meaningful hematologic and solid tumor therapies that address unmet needs of patients facing unique challenges and difficult odds," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development of Jazz Pharmaceuticals. "Our dedication to patient-centered research, through internal R&D capabilities and strategic external partnerships, will help ensure a greater number of people benefit from the development of new treatment options, as well as expand our presence in oncology."

Research findings to be presented include new data from Jazz's marketed products, Vyxeos®/Vyxeos® Liposomal (daunorubicin and cytarabine), also known as CPX-351, and Defitelio® (defibrotide sodium), as well as late-stage investigational drug candidates, JZP-458 and lurbinectedin.

Highlights from Jazz and its partners at ASCO will include:

- A poster discussion presentation featuring final results from a five-year analysis of a pivotal Phase 3 study comparing outcomes for Vyxeos (CPX-351) versus 7+3 in older adults with newly diagnosed high-risk/secondary acute myeloid leukemia (sAML)
- A poster presentation from Jazz's partner, PharmaMar, featuring a pooled safety analysis of lurbinectedin from the Phase 2 basket study

Highlights from Jazz at EHA will include:

- A poster presentation featuring final results from a five-year analysis of a pivotal Phase 3 study comparing outcomes for CPX-351 versus 7+3 in older adults with newly diagnosed high-risk/sAML
- A poster presentation of outcomes in adult and pediatric patients from an observational registry study of defibrotide treatment for veno-occlusive disease/sinusoidal obstruction syndrome after hematopoietic cell transplantation

All ASCO virtual poster presentations and poster discussion presentations will be available on-demand to registered participants for 180 days beginning May 29, 2020. The Jazz-supported and partner poster and poster discussion presentations covering CPX-351, JZP-458 and lurbinectedin at the ASCO Annual Meeting are:

Vyxeos Poster and Poster Discussion Presentations

Presentation Title	Author	Poster Number / Abstract Link
Phase 1B Study of CPX-351 Lower-Intensity Therapy Plus Venetoclax as First-Line Treatment for Patients with Acute Myeloid Leukemia Who Are Unfit for Intensive Chemotherapy (trial in progress)	Lin et al.	Poster Number: 340 Abstract Link
Outcomes in Older Patients with High-Risk/Secondary Acute Myeloid Leukemia Who Achieved Remission with CPX-351 Versus 7+3 But Did Not Undergo Transplant: Phase 3 Exploratory Analysis	Lin et al.	Poster Number: 310 Abstract Link
Five-Year Final Results of a Phase III Study of CPX-351 Versus 7+3 in Older Adults with Newly Diagnosed High-Risk/Secondary Acute Myeloid Leukemia (Poster discussion)	Lancet et al.	Poster Number: 283 Abstract Link

Children's Oncology Group Poster Presentation

Presentation Title	Author	Poster Number / Abstract Link
Change in Cardiac Function with CPX-351 in Relapsed Pediatric Acute Myeloid Leukemia Patients: A Children's Oncology Group (COG) Report from AAML 1421	Leger et al.	Poster Number: 419 Abstract Link

JZP-458 Poster Presentation

Presentation Title	Author	Poster Number / Abstract Link
Phase II/III Study of JZP-458 in Patients with Acute Lymphoblastic Leukemia/Lymphoblastic Lymphoma Who Are Hypersensitive to *E. coli*-Derived Asparaginases (trial in progress)	Maese L, et al.	Poster Number: 341 Abstract Link

Lurbinectedin Poster and Poster Discussion Presentations

Presentation Title	Author	Poster Number / Abstract Link

Pooled Safety Analysis of Single-Agent Lurbinectedin Versus Topotecan	Leary et al.	Poster Number: 365 Abstract Link
Lurbinectedin in Combination with Irinotecan in Patients with Advanced Solid Tumors (Poster discussion)	Ponce et al.	Poster Number: 244 Abstract Link

The Jazz-supported poster presentations and publications covering CPX-351 and defibrotide at the 25th Annual Congress of the EHA are:

Vyxeos Liposomal Poster Presentations

Presentation Title	Author	Abstract Code / Abstract Link / Session Title
Outcomes in Older Patients with High-Risk/Secondary Acute Myeloid Leukemia Who Achieved Remission with CPX-351 Versus 7+3 But Did Not Undergo Transplant: Phase 3 Exploratory Analysis	Lin et al.	Abstract Code: EP562 Abstract Link Session Title: Acute myeloid leukemia – clinical
Five-Year Final Results of a Phase 3 Study of CPX-351 Versus 7+3 in Older Adults with Newly Diagnosed High-Risk/Secondary Acute Myeloid Leukemia	Lancet J, et al.	Abstract Code: EP556 Abstract Link Session Title: Acute myeloid leukemia – clinical
Exploratory Analysis of the Efficacy and Safety of CPX-351 Versus 7+3 by European Leukemia Net (ELN) 2017 Risk Groups in a Phase 3 Study of Older Adults with High-Risk/Secondary Acute Myeloid Leukemia	Prebet et al.	Abstract Code: EP571 Abstract Link Session Title: Acute myeloid leukemia – clinical
Analysis of Treatments and Outcomes for Patients with *De Novo* AML, Therapy-Related AML and Secondary AML (Prior MDS and CMML) Diagnosed in England Between 2011-2016 Using Hospital Episode Statistics	Legg et al.	Abstract Code: EP605 Abstract Link Session Title: Acute myeloid leukemia – clinical
Patient Experiences with CPX-351 Versus Conventional Induction Regimens: An Analysis of Patient-Reported Outcomes Data from a Prospective Trial	LeBlanc et al.	Abstract Code: PB1832 Abstract Link Publication only
Therapeutic Targeting of DNA-Dependent Protein Kinase Catalytic Subunit (DNA-PKcs) with M3814 in Combination with CPX-351 In TP53wt and TP53mut AML	Nishida et al.	Abstract Code: EP473 Abstract Link Session Title: Acute myeloid leukemia - Biology & Translational Research

Defibrotide Poster Presentation

Presentation Title	Author	Abstract Code / Abstract Link / Session Title
Defibrotide Treatment for Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome after Hematopoietic Cell Transplantation: Outcomes in Adult and Pediatric Patients from an Observational Registry Study	Mohty et al.	Abstract Code: EP1377 Abstract Link Session Title: Stem cell transplantation – clinical

About Vyxeos® (daunorubicin and cytarabine)

In the U.S., 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 acute myeloid leukemia (AML): newly diagnosed therapy-related AML (t-AML) and AML with myelodysplasia-related changes (AML-MRC). In Europe, Vyxeos® Liposomal (daunorubicin/cytarabine) is indicated for the treatment of adults with newly diagnosed, therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC). Backed by a robust clinical development program including Phase 3 data, Vyxeos is currently approved in more than 30 countries, and Jazz continues to work with regulatory authorities worldwide to bring this innovative therapy to appropriate patients.

Important Safety Information for Vyxeos Liposomal

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

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

Vyxeos Liposomal 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 Liposomal. Patients should tell the doctor about new onset fever or symptoms of infection or if they notice signs of bruising or bleeding.

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

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

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 Liposomal 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. Food and Drug Administration (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. Defitelio is not approved for the prevention of VOD.

Please see full Prescribing Information for Defitelio.

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)

Defibrotide is currently being investigated in two Phase 2 trials for the prevention of acute Graft-versus-Host-Disease (aGvHD) and the prevention of neurotoxicity in patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) receiving CAR T-cell therapy.

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
Investigational drug, JZP-458 is a recombinant *Erwinia* asparaginase that uses a novel *Pseudomonas fluorescens* expression platform. It is being investigated 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. The FDA has yet to review JZP-458, and its safety and effectiveness have not yet been established. JZP-458 was granted Fast Track designation by the FDA in October 2019.

About Lurbinectedin (PM1183)
Lurbinectedin (PM1183) is a synthetic compound currently under clinical investigation. It is a selective inhibitor of the oncogenic transcription programs on which many tumors are particularly dependent. Together with its effect on cancer cells, lurbinectedin inhibits oncogenic transcription in tumor-associated macrophages, downregulating the production of cytokines that are essential for the growth of the tumor. Transcriptional addiction is an acknowledged target in those diseases, many of them lacking other actionable targets. Lurbinectedin was discovered and is owned by Pharma Mar, S.A. (PharmaMar). As previously announced in December 2019, PharmaMar and Jazz Pharmaceuticals have entered into an exclusive license agreement, which became effective in January 2020, granting Jazz U.S. commercialization rights to lurbinectedin. Lurbinectedin is an investigational drug under review by the FDA, and its safety and effectiveness have not yet been established.

About Jazz Pharmaceuticals plc
Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a global biopharmaceutical company dedicated to developing life-changing medicines for people with serious diseases — often with limited or no options. We have a diverse portfolio of marketed medicines and novel product candidates, from early- to late-stage development, in key therapeutic areas. Our focus is in neuroscience, including sleep medicine and movement disorders, and in oncology, including hematologic and solid tumors. We actively explore new options for patients including novel compounds, small molecule advancements, biologics and innovative delivery technologies. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in more

than 90 countries. For more information, please visit www.jazzpharmaceuticals.com and follow @JazzPharma on Twitter.

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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