Jazz to Present New Data at ASCO and EHA 2021 Meetings Showcasing Clinical Advances Across Hematology and Oncology Portfolio

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Findings continue to demonstrate Jazz's leadership in developing treatments for difficult-to-treat conditions where few options exist

DUBLIN, May 20, 2021 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that it and its investigator sponsors will present four new abstracts at the virtual American Society of Clinical Oncology (ASCO) Annual Meeting from June 4 – June 8, 2021 and five abstracts at the virtual 26th Annual Congress of the European Hematology Association (EHA2021) from June 9 –16, 2021. Research findings to be presented include new data on Zepzelca® (lurbinectedin), Vyxeos®/Vyxeos® Liposomal (daunorubicin and cytarabine), also known as JZP351 (formerly known as CPX-351), and Defitelio® (defibrotide sodium).

"The unmet needs of patients facing unique challenges and difficult odds drive our research and development objectives, and it's critical for us to continue enhancing our understanding of our existing therapies so that the greatest number of patients can benefit from our innovative medicines," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development and chief medical officer of Jazz Pharmaceuticals. "We are excited to build upon our growing oncology portfolio and make meaningful strides for more people living with rare or complex hematologic and oncologic diseases."

Highlights from Jazz and its investigational sponsors at the congresses will include:

- A poster presentation at ASCO featuring results from the Phase 1/2 study evaluating the combination of lurbinectedin plus irinotecan in women with endometrial carcinoma.
- A poster presentation featuring preliminary results from V-FAST, a Phase 1b clinical trial evaluating JZP351 in combination with one of three targeted agents (venetoclax, midostaurin or enasidenib) in adults with previously untreated acute myeloid leukemia (AML) who are considered fit for intensive chemotherapy. These data will be presented at both ASCO and EHA.

All ASCO virtual poster presentations and poster discussion presentations will be available on-demand to registered participants for 180 days beginning June 4, 2021. The Jazz-supported and investigational sponsor presentations and publications covering lurbinectedin and JZP351 (formerly known as CPX-351) at the ASCO Annual Meeting are:

Lurbinectedin Presentations

Presentation Title	Author	Poster Number / Abstract Link
Phase 1/2 Study of Lurbinectedin in Combination with Irinotecan in Patients with Endometrial Carcinoma	Sanchez-Simon I, et al.	Poster Number: 5586 Abstract Link
Treatment Patterns of Patients with Small Cell Lung Cancer in Second and Third-Line Therapy Settings	Prince P, et al.	*Publication only*
Overall Survival of Small Cell Lung Cancer Patients Initiating Anti-Cancer Treatment	Estrin A, et al.	*Publication only*

JZP351 (formerly known as CPX-351) Poster Presentation

Presentation Title	Author	Poster Number / Abstract Link
Preliminary Results of V-FAST, a Phase 1B Master Trial to Investigate CPX-351 Combined with Targeted Agents in Newly Diagnosed AML	Pullarkat V, et al.	Poster Number: 7026 Abstract Link

The poster presentations and publications covering JZP351 (formerly known as CPX-351) and defibrotide sodium at EHA2021 virtual congress are:

JZP351 (formerly known as CPX-351) Presentations

Presentation Title	Author	Abstract Code / Abstract Link
Preliminary Results of V-FAST, a Phase 1B Master Trial to Investigate CPX-351 Combined with Targeted Agents in Newly Diagnosed AML	Pullarkat V, et al.	Poster Number: EP442 Abstract Link

Rapid Genomic Profiling of AML Patients with Myelodysplasia-related Cytogenetic Aberrations *(investigator sponsored trial)*	Dolnik A, et al.	Poster Number: EP402 Abstract Link
Pilot Study of CPX-351 in Adults with Relapsed/Refractory Acute Lymphoblastic Leukemia *(investigator sponsored trial)*	Shah BD, et al.	*Publication only*

Defibrotide Sodium Poster Presentations

Presentation Title	Author	Abstract Code / Abstract Link
Incidence of Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) After Hematopoietic Cell Transplantation (HCT) in England Based on Healthcare Resource Group Codes and Hospital Episode Statistics© Data	Angus J, et al.	Poster Number: EP1258 Abstract Link
EBMT Pass Registry Outcomes of Defibrotide-Treated Patients by Timing of Onset of VOD/SOS After HCT	Mohty M, et al.	Poster Number: EP1238 Abstract Link

About Vyxeos®/Vyxeos® Liposomal (daunorubicin and cytarabine), also known as JZP 351
Vyxeos is a liposomal combination of daunorubicin, an anthracycline topoisomerase inhibitor, and cytarabine, a nucleoside metabolic inhibitor.

In the U.S., *Vyxeos* is indicated for the treatment of newly-diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) in adults and pediatric patients 1 year and older.[1]

More information about Vyxeos in the United States, including Full Prescribing Information, BOXED Warning and Medication Guide, is available here.

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.

The full Summary of Product Characteristics of Vyxeos Liposomal in Europe is available here.

Important Safety Information for VYXEOS/VYXEOS LIPOSOMAL
WARNING: 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.

Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Jazz Pharmaceuticals at 1-800-520-5568.

About Zepzelca® (lurbinectedin)
Disclaimer: All information about Zepzelca is intended for a U.S.-audience only.
Zepzelca is an alkylating drug that binds guanine residues within DNA. This triggers a cascade of events that can affect the activity of DNA binding proteins, including some transcription factors, and DNA repair pathways, resulting in disruption of the cell cycle and eventual cell death.[2]

Zepzelca for injection 4 mg is a prescription medicine used to treat adults with a kind of lung cancer called small cell lung cancer that has spread to other parts of the body (metastatic) and who have received treatment with chemotherapy that contains platinum, and it did not work or is no longer working. *Zepzelca* is approved based on response rate and how long the response lasted. Additional studies will further evaluate the benefit of *Zepzelca* for this use.

Important Safety Information for ZEPZELCA

Before receiving ZEPZELCA, tell your healthcare provider about all of your medical conditions, including if you:

- have liver or kidney problems.
- are pregnant or plan to become pregnant. ZEPZELCA can harm your unborn baby.
  Females who are able to become pregnant:
- Your healthcare provider should do a pregnancy test before you start treatment with ZEPZELCA.
- You should use effective birth control (contraception) during treatment with and for 6 months after your final dose of ZEPZELCA.
- Tell your healthcare provider right away if you become pregnant or think that you are pregnant during treatment with ZEPZELCA.

Males with female partners who are able to become pregnant should use effective birth control during treatment with and for 4 months after your final dose of ZEPZELCA.

- are breastfeeding or plan to breastfeed. It is not known if ZEPZELCA passes into your breastmilk. Do not breastfeed during treatment with ZEPZELCA and for 2 weeks after your final dose of ZEPZELCA. Talk to your healthcare provider about the best way to feed your baby during treatment with ZEPZELCA.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Certain other medicines may affect how ZEPZELCA works.

What should I avoid while using ZEPZELCA?

Avoid eating or drinking grapefruit, or products that contain grapefruit juice during treatment with ZEPZELCA.

ZEPZELCA can cause serious side effects, including:

- **Low blood cell counts.** Low blood counts including low neutrophil counts (neutropenia) and low platelet counts (thrombocytopenia) are common with ZEPZELCA, and can also be severe. Some people with low white blood cell counts may get fever, or an infection throughout the body (sepsis), that can cause death. Your healthcare provider should do blood tests before you receive each treatment with ZEPZELCA to check your blood cell counts.
  Tell your healthcare provider right away if you develop:
- fever or any other signs of infection
- unusual bruising or bleeding
- tiredness
- pale colored skin
- **Liver problems.** Increased liver function tests are common with ZEPZELCA, and can also be severe. Your healthcare provider should do blood tests to check your liver function before you start and during treatment with ZEPZELCA.
  Tell your healthcare provider right away if you develop symptoms of liver problems including:
- loss of appetite
- nausea or vomiting
- pain on the right side of your stomach area (abdomen)

Your healthcare provider may temporarily stop treatment, lower your dose, or permanently stop ZEPZELCA if you develop low blood cell counts or liver problems during treatment with ZEPZELCA.

The most common side effects of ZEPZELCA include:

- tiredness
- low white and red blood cell counts
- increased kidney function blood test (creatinine)
- increased liver function blood tests
- increased blood sugar (glucose)
- nausea
- decreased appetite
- muscle and joint (musculoskeletal) pain
- low level of albumin in the blood
- constipation
- trouble breathing
- low levels of sodium and magnesium in the blood
- vomiting
- cough
- diarrhea

These are not all of the possible side effects of ZEPZELCA.

Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Jazz Pharmaceuticals at 1-800-520-5568.

More information about Zepzelca, including Full Prescribing Information and Patient Information, is available here.

ZEPZELCA is a trademark of PharmaMar, S.A. used by Jazz Pharmaceuticals under license.

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, and it is indicated 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 the United States.

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

The full Summary of Product Characteristics of Defitelio in Europe is available here.

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.

About Jazz Pharmaceuticals plc
Jazz Pharmaceuticals plc (NASDAQ: JAZZ) is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing life-changing medicines for people with serious diseases – often with limited or no therapeutic options. We have a diverse portfolio of marketed medicines and novel product candidates, from early- to late-stage development, in neuroscience and oncology. We actively explore new options for patients including novel compounds, small molecules and biologics, and through cannabinoid science and innovative delivery technologies. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries. For more information, please visit www.jazzpharmaceuticals.com and follow @JazzPharma on Twitter.

Caution Concerning Forward-Looking Statements
This press release contains forward-looking statements, including, but not limited to, statements related to potentially addressing patient needs with our oncology portfolio and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' 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 pharmaceutical product development, and other risks and uncertainties affecting Jazz Pharmaceuticals 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 Jazz Pharmaceuticals' Annual Report on Form 10-K for the year ended December 31, 2020 and future filings and reports by Jazz Pharmaceuticals. Other risks and uncertainties of which Jazz Pharmaceuticals is not currently aware may also affect Jazz Pharmaceuticals' 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 Jazz Pharmaceuticals on its website or otherwise. Jazz Pharmaceuticals 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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[1] Vyxeos (daunorubicin and cytarabine) Prescribing Information. Palo Alto, CA: Jazz Pharmaceuticals, Inc.
[2] ZEPZELCA (lurbinectedin) Prescribing Information. Palo Alto, CA: Jazz Pharmaceuticals, Inc.

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