Jazz Pharmaceuticals to Present Data Showcasing Clinical Advancements Across Oncology Portfolio at 2022 ASCO and EHA Meetings

May 12, 2022

Key data includes ASCO oral presentation of data from the Rylaze® (asparaginase erwinia chrysanthemi (recombinant)-rywn) Phase 2/3 trial in Acute Lymphoblastic Leukemia or Lymphoblastic Lymphoma

DUBLIN, May 12, 2022 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the Company and its partners will present seven abstracts at the American Society of Clinical Oncology (ASCO) Annual Meeting from June 3 – June 7, 2022, and eight abstracts at the 27th Annual Congress of the European Hematology Association (EHA) from June 9 –12, 2022. Research findings to be presented include data on *Rylaze*/JZP458, Zepzelca® (lurbinectedin), Defitelio® (defibrotide sodium) and Vyxeos®/Vyxeos® Liposomal (daunorubicin and cytarabine), also known as JZP351.

"As part of Jazz's commitment to explore potential new applications of our oncology medicines to address critical needs, we continue to advance programs that could impact difficult-to-treat therapeutic areas," said Rob Iannone, M.D., M.S.C.E., executive vice president, global head of research and development of Jazz Pharmaceuticals. "Our growing, early-stage pipeline, combined with ongoing Jazz-sponsored and partner research across our portfolio, is making significant progress when it comes to addressing unmet patient needs in cancers that have historically lacked scientific advancements."

Highlights from Jazz and its investigational sponsors at the congresses feature data for our medicines across a range of solid tumors and hematological malignancies, including:

- An oral presentation at ASCO featuring results from Cohort 1 of a Phase 2/3 trial conducted with the Children's Oncology Group, evaluating the efficacy and safety of *Rylaze* administered intramuscularly (IM) on a Monday/Wednesday/Friday dosing schedule, for patients living with acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL).
- Two poster presentations for JZP351 including preliminary results from Arm B (JZP351 in combination with midostaurin) from the Phase 1b V-FAST trial in adults with previously untreated FLT3-mutated acute myeloid leukemia (AML) and data from the Phase 1b trial evaluating lower-intensity JZP351 + venetoclax in adults with newly diagnosed AML who are unfit for intensive chemotherapy. These data will be presented at both ASCO and EHA.
- Four poster presentations at ASCO evaluating *Zepzelca* in a range of small cell lung cancer (SCLC) settings, both as a monotherapy and in combination with other therapies, and in *BRCA*1/2-associated metastatic breast cancer.

The Jazz-supported presentations at the 2022 ASCO Annual Meeting are:

Rylaze Presentations

Presentation Title	Author	Presentation Details
Efficacy and safety of intramuscular (IM) recombinant Erwinia asparaginase in acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL): The Children's Oncology Group (COG) AALL1931 study	Maese L, et al.	**Type**: Oral presentation **Session:** Oral Abstract Session/ Hematologic Malignancies—Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date**: June 7 at 10:45 a.m. EDT **Abstract number:** 7001 Abstract Link

Zepzelca Presentations

Presentation Title	Author	Presentation Details
Analysis of patients with relapsed small cell lung cancer (SCLC) receiving single-agent lurbinectedin in the phase 3 ATLANTIS trial	Navarro A, et al.	**Type:** Poster **Session:** Lung Cancer – Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date:** June 6 at 9:00 a.m. EDT **Abstract number:** 8524 Abstract link
Efficacy and safety of lurbinectedin as second-line therapy in Chinese patients with small cell lung cancer: Preliminary results of a phase 1 study	Cheng Y, et al.	**Type:** Poster **Session:** Lung Cancer – Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date:** June 6 at 9:00 a.m. EDT **Abstract number:** 8580 Abstract link

A phase 1/2 trial of lurbinectedin (L) in combination with pembrolizumab (P) in relapsed small cell lung cancer (SCLC): The LUPER study	Calles Blanco A, et al.	**Type:** Poster **Session:** Lung Cancer – Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date:** June 6 at 9:00 a.m. EDT **Abstract number:** 8581 Abstract link
Lurbinectedin in patients with pretreated BRCA1/2-associated metastatic breast cancer: Results from a phase II basket study	Boni V, et al.	**Type:** Poster **Session:** Breast Cancer – Metastatic **Date:** June 6 at 9:00 a.m. EDT **Abstract number:** 1092 Abstract link

Vyxeos Presentations

Presentation Title	Author	Presentation Details
V-FAST master trial: Preliminary results of treatment with CPX-351 plus midostaurin in adults with newly diagnosed FLT3-mutated acute myeloid leukemia	McCloskey J, et al.	**Type:** Poster **Session:** Hematologic Malignancies – Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date:** June 4 at 9:00 a.m. EDT **Abstract number**: 7043 Abstract link
Lower-intensity CPX-351 + venetoclax for patients with newly diagnosed AML who are unfit for intensive chemotherapy	Uy G.L. et al.	**Type:** Poster **Session:** Hematologic Malignancies – Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date:** June 4 at 9:00 a.m. EDT **Abstract number:** 7031 Abstract link

The Jazz-supported presentations at the EHA 27th Congress are:

Vyxeos Liposomal Presentations

Presentation Title	Author	Presentation Details
Lower-intensity CPX-351 + venetoclax for patients with newly diagnosed acute myeloid leukemia who are unfit for intensive chemotherapy	Uy L. G. et al.	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P515 Abstract link
CPX-351 treatment for acute myeloid leukemia in England: Real-world outcomes in adults aged <60 years versus ≥60 years	Legg A. et al.	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P513 Abstract link
V-FAST master trial: Preliminary results of treatment with CPX-351 plus midostaurin in adults with newly diagnosed *FLT3*-mutated acute myeloid leukemia	McCloskey J, et al	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P514 Abstract link
Real life experience using front-line CPX-351 for therapy-related and AML-MRC: results from the Spanish PETHEMA registry (IST)	Bernal T, et al	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P508 Abstract link
A randomised comparison of CPX-351 and FLAG-Ida in high risk acute myeloid leukaemia. Results from the NCRI AML19 trial	Russel N, et al	**Type:** Oral presentation **Date:** June 11 **Abstract number:** S128 Abstract link

Defitelio Presentations

Presentation Title	Author	Presentation Details

Veno-occlusive disease/sinusoidal obstruction syndrome (VOD/SOS) after autologogous hematopoietic cell transplantion (HCT): Outcomes of defibrotide-treated adult patients from the DefiFrance study	Mohty M, et al	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P1355 Abstract link
A systematic literature review (SLR) of the manifestations of veno-occlusive disease/sinusoidal obstruction syndrome (VOD/SOS) after hematopoietic cell transplant (HCT) in adults versus children	Angus J, et al.	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P1354 Abstract link

Asparaginase Presentations

Presentation Title	Author	Presentation Details
Phase 1 trial of pegcrisantaspase in combination with venetoclax in adults with relapsed or refractory acute myeloid leukemia (R/R AML) – Safety, efficacy and PK/PD in the first two cohorts	Liu Y, et al	**Type:** Poster **Date:** June 10 at 16:30 CEST **Abstract number:** P527 Abstract link

All ASCO virtual poster presentations and poster discussion presentations will be available on-demand to registered participants for 180 days beginning June 3, 2022. EHA presentations will be available on-demand beginning June 20, 2022.

About Zepzelca® (lurbinectedin)
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 RYLAZE® (asparaginase erwinia chrysanthemi (recombinant)-rywn)
RYLAZE, also known as JZP458, is approved in the U.S. for use as a component of a multi-agent chemotherapeutic regimen for the treatment of acute lymphoblastic leukemia (ALL) and lymphoblastic lymphoma (LBL) in adult and pediatric patients one month or older who have developed hypersensitivity to E. coli-derived asparaginase. RYLAZE has orphan drug designation for the treatment of ALL/LBL in the United States. RYLAZE is a recombinant erwinia asparaginase that uses a novel Pseudomonas fluorescens expression platform. JZP458 was granted Fast Track designation by the U.S. Food and Drug Administration (FDA) in October 2019 for the treatment of this patient population. RYLAZE was approved as part of the Real-Time Oncology Review program, an initiative of the FDA's Oncology Center of Excellence designed for efficient delivery of safe and effective cancer treatments to patients.

The full U.S. Prescribing Information for RYLAZE is available here.

Important Safety Information for Rylaze

RYLAZE should not be given to people who have had:

- Serious allergic reactions to RYLAZE
- Serious swelling of the pancreas (stomach pain), serious blood clots, or serious bleeding during previous asparaginase treatment

RYLAZE may cause serious side effects, including:

- Allergic reactions (a feeling of tightness in your throat, unusual swelling/redness in your throat and/or tongue, or trouble

breathing), some of which may be life-threatening
- Swelling of the pancreas (stomach pain)
- Blood clots (may have a headache or pain in leg, arm, or chest)
- Bleeding
- Liver problems

Contact your doctor immediately if any of these side effects occur.

Some of the most common side effects with RYLAZE include: liver problems, nausea, bone and muscle pain, tiredness, infection, headache, fever, allergic reactions, fever with low white blood cell count, decreased appetite, mouth swelling (sometimes with sores), bleeding, and too much sugar in the blood.

RYLAZE can harm your unborn baby. Inform your doctor if you are pregnant, planning to become pregnant, or nursing. Females of reproductive potential should use effective contraception (other than oral contraceptives) during treatment and for 3 months following the final dose. Do not breastfeed while receiving RYLAZE and for 1 week after the final dose.

Tell your healthcare provider if there are any side effects that are bothersome or that do not go away.

These are not all the possible side effects of RYLAZE. For more information, ask your healthcare provider.

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 (1-800-332-1088).

About Vyxeos®/Vyxeos® Liposomal (daunorubicin and cytarabine), also known as JZP351
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 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. Within these therapeutic areas, we are identifying new options for patients by actively exploring small molecules and biologics, and through innovative delivery technologies and cannabinoid science. 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, 2021 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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