Jazz Pharmaceuticals to Present Data Showcasing Clinical Advances Across Hematology/Oncology at ASH 2021 Annual Meeting

November 4, 2021

Sixteen new abstracts to be presented, including the first presentation of data from the Rylaze™ (asparaginase erwinia chrysanthemi (recombinant)-rywn) Phase 2/3 study that supported U.S. FDA approval earlier this year

DUBLIN, Nov. 4, 2021 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that 16 new data abstracts from across its hematology/oncology development program will be presented at the 63rd American Society of Hematology (ASH) Annual Meeting, which will be held December 11-14, 2021. This includes five presentations from investigator-sponsored trials and three presentations from collaboration studies with The University of Texas MD Anderson Cancer Center (MD Anderson).

"The data at ASH demonstrates Jazz's focus on making a difference for people living with rare forms of leukemias and blood cancers, both through the development of new treatment options as well as further evaluating our currently approved medicines," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development and chief medical officer of Jazz Pharmaceuticals. "Our support of several investigator-sponsored and collaboration trials exemplifies our commitment to working with experts to enable studies beyond our own company-sponsored trials, and to identifying new treatment options for patients through a variety of means."

Highlights at ASH include:

- A poster presentation sharing, for the first time, data from the Phase 2/3 study of *Rylaze* in patients with acute lymphoblastic leukemia (ALL)/lymphoblastic lymphoma (LBL) who developed hypersensitivity or silent inactivation to a long-acting *E. coli*–derived asparaginase.
- Results for Vyxeos® (daunorubicin and cytarabine) in acute myeloid leukemia (AML) including an oral presentation from a real-world evidence study of *Vyxeos* use in newly diagnosed patients and a poster presentation from a Phase 1b study of lower-dose *Vyxeos* in combination with venetoclax in patients with AML who are unfit for intensive chemotherapy.
- Data for *Vyxeos* use in new patient populations, including oral presentations of two studies of *Vyxeos* as treatment in higher risk Myelodysplastic Syndrome (MDS).
- A poster presentation with final results from a real-world evidence study, DEFIFrance, of Defitelio® (defibrotide sodium) treatment in adults with severe or very severe veno-occlusive disease/sinusoidal obstruction syndrome after hematopoietic cell transplantation.

The ASH abstracts are available online starting today, November 4 at https://ash.confex.com/ash/2021/webprogram/start.html.

ASH will be held as a hybrid conference virtually and in-person in Atlanta, GA at the Georgia World Congress Center. A full list of Jazz and investigator-sponsored presentations follows below:

Rylaze Presentations

Presentation Topic	Author	Date / Time (EST) / Session Title / Presentation Number
Initial Results from a Phase 2/3 Study of Recombinant Erwinia Asparaginase (JZP458) in Patients with Acute Lymphoblastic Leukemia (ALL)/Lymphoblastic Lymphoma (LBL) Who are Allergic/Hypersensitive to E. Coli–Derived Asparaginases	Luke Maese et al.	• **Type:** Poster • **Number:** 2307 • **Session:** 614. Acute Lymphoblastic Leukemias: Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster II • **Date/Time:** Sunday, December 12, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5

Vyxeos Presentations

Presentation Topic	Author	Date / Time (EST) / Session Title / Presentation Number
A Pilot Study of CPX-351 (Vyxeos®) for Transplant Eligible, Higher Risk Patients with Myelodysplastic Syndrome	Meagan A. Jacoby et al.	• **Type:** Oral Presentation • **Number:** 540 • **Session:** 637. Myelodysplastic Syndromes – Clinical and Epidemiological: Treatment of HIgh Risk and Relapsed/Refractory Myelodysplastic Syndrome • **Date/Time:** Sunday, December 12, 2021: 4:30

		PM-6:00 PM • **Location:** B211-B212
Real-World Experience of CPX-351 As First-Line Treatment in 188 Patients with Acute Myeloid Leukemia	Christina Rautenberg et al.	• **Type:** Oral Presentation • **Number:** 33 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Innovative induction regimens in AML: data from real life and clinical trials • **Date/Time:** Saturday, December 11, 2021: 9:30 AM-11:00 AM • **Location:** B405-B407
CPX 351 As First Line Treatment in Higher Risk MDS. a Phase II Trial By the GFM	Pierre Peterlin et al.	• **Type:** Oral Presentation • **Number:** 243 • **Session:** 637. Myelodysplastic Syndromes – Clinical and Epidemiological: Treatment of High Risk Myelodysplastic Syndrome • **Date/Time:** Saturday, December 11, 2021: 2:00 PM-3:30 PM • **Location:** B207-B208
Preliminary Results by Age Group of Treatment with CPX-351 Plus Venetoclax in Adults with Newly Diagnosed AML: Subgroup Analysis of the V-FAST Phase 1b Master Trial	Vinod Pullarkat et al.	• **Type:** Poster • **Number:** 1268 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster I • **Date/Time:** Saturday, December 11, 2021: 5:30 PM-7:30 PM • **Location:** Hall B5
Phase 1b Study of Lower-dose CPX-351 Plus Venetoclax As First-line Treatment for Patients with AML Who Are Unfit for Intensive Chemotherapy: Preliminary Safety and Efficacy Results	Geoffrey L. Uy et al.	• **Type:** Poster • **Number:** 2316 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster II • **Date/Time:** Sunday, December 12, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5
Real-World Study of the Treatment Patterns of Patients Diagnosed with Therapy-Related AML or AML-MRC in England between 2013 and 2020 Using the Cancer Analysis System Database	Alex Legg et al.	• **Type:** Poster • **Number:** 1248 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster I • **Date/Time:** Saturday, December 11, 2021: 5:30 PM-7:30 PM • **Location:** Hall B5
Real-World Study of CPX-351 Treatment Outcomes for Acute Myeloid Leukemia (AML) in England	Alex Legg et al.	• **Type:** Poster • **Number:** 2310 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster II • **Date/Time:** Sunday, December 12, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5
Updated Results of a Phase 1/2 Study of Lower Dose CPX-351 for Patients with Int-2 or High Risk IPSS Myelodysplastic Syndromes and Chronic Myelomonocytic Leukemia after Failure to Hypomethylating Agents	Guillermo Montalban-Bravo et al.	• **Type:** Poster • **Number:** 3674 • **Session:** 637. Myelodysplastic Syndromes — Clinical and Epidemiological: Poster III

		• **Date/Time:** Monday, December 13, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5
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)	Daniel Rivera et al.	• **Type:** Poster • **Number:** 2323 • **Session:** 615. Acute Myeloid Leukemias: Commercially Available Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster II • **Date/Time:** Sunday, December 12, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5
A Phase II Study of CPX-351 plus Venetoclax in Patients with Relapsed/Refractory (R/R) or Newly Diagnosed Acute Myeloid Leukemia (AML)	Kunhwa Kim et al.	• **Type:** Poster • **Number:** 1275 • **Session:** 616. Acute Myeloid Leukemias: Investigational Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster I • **Date/Time:** Saturday, December 11, 2021: 5:30 PM-7:30 PM • **Location:** Hall B5

Defitelio Presentations

Presentation Topic	**Author**	**Date / Time (EST) / Session Title / Presentation Number**
A Phase 3, Randomized, Adaptive Study of Defibrotide (DF) Vs Best Supportive Care (BSC) for the Prevention of Hepatic Veno-occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) in Patients (pts) Undergoing Hematopoietic Cell Transplantation (HCT): Preliminary Results	Stephan A. Grupp et al.	• **Type:** Oral Presentation • **Number:** 749 • **Session:** 721. Allogeneic Transplantation: Conditioning Regimens, Engraftment and Acute Toxicities; Prevention and Management of Complications • **Date/Time:** Monday, December 13, 2021; 2:45 PM – 4:15 PM EST • **Presentation Time:** 3:45 PM EST • **Location:** Thomas Murphy Ballroom 3-4
Final Long-term Results from the DEFIFrance Registry Study: Efficacy and Safety of Defibrotide for the Treatment of Severe/Very Severe Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome after Hematopoietic Cell Transplantation	Mohamad Mohty et al.	• **Type:** Poster • **Number:** 1789 • **Session:** 721. Allogeneic Transplantation: Conditioning Regimens, Engraftment and Acute Toxicities: Poster I • **Date/Time:** Saturday, December 11, 2021; 5:30 PM – 7:30 PM EST • **Location:** Hall B5
Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome Without Hematopoietic Cell Transplantation in a Real-World Population in the United States: Patient Characteristics, Prior Treatment Patterns, and Time to Diagnosis	Xue Wang et al.	• **Type:** Poster • **Number:** 1946 • **Session:** 904. Outcomes Research —Non-Malignant Conditions: Poster I • **Date/Time:** Saturday, December 11, 2021; 5:30 PM – 7:30 PM EST • **Location:** Hall B5
Defibrotide Therapy for Sars CoV2 Acute Respiratory Distress Syndrome	Gregory Yanik et al.	• **Type:** Poster • **Number:** 3237 • **Session:** 332. Anticoagulation and Antithrombotic Therapies: Poster III • **Date/Time:** Monday, December 13, 2021: 6:00 PM-8:00 PM • **Location:** Hall B5
Use of Defibrotide in Patients with COVID-19 Pneumonia; Results of the Defi-VID19 Phase 2 Trial	Annalisa Ruggeri et al.	• **Type:** Oral Presentation • **Number:** 672 • **Session**: 332. Anticoagulation and

		Antithrombotic Therapies • **Date/Time**: Monday, December 13, 2021: 2:45 PM-4:15 PM • **Location**: B401-B402

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) or lymphoblastic lymphoma (LBL) in pediatric and adult patients one month and 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.

Important Safety Information

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.

The full U.S. Prescribing Information for Rylaze is available at: http://pp.jazzpharma.com/pi/rylaze.en.USPI.pdf

About Vyxeos® (daunorubicin and cytarabine)

Vyxeos is a liposomal combination of daunorubicin, an anthracycline topoisomerase inhibitor, and cytarabine, a nucleoside metabolic inhibitor, that 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. For more information about Vyxeos in the United States, please visit https://vyxeos.com.

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).

Important Safety Information for Vyxeos®

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
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.

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