Jazz Pharmaceuticals to Highlight Hematology Research at ASH 2017 Annual Meeting

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Nineteen abstracts accepted for three Jazz hematology/oncology products

DUBLIN, Nov. 1, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that thirteen abstracts spanning the company's hematology/oncology portfolio will be presented at the 59th American Society of Hematology (ASH) Annual Meeting in Atlanta from December 9-12, with an additional six abstracts accepted for online publication only in ASH's weekly medical journal *Blood* Online.

"The breadth of data presented at ASH reflects our commitment to addressing the clinical needs of patients with blood cancers and with complications of stem cell transplantation by advancing the science behind Vyxeos, Defitelio and Erwinaze," said Karen Smith, M.D., Ph.D., executive vice president, research and development and chief medical officer of Jazz Pharmaceuticals. "Jazz continues to expand its hematology/oncology portfolio and we are grateful to the patients and health care professionals who participate in our clinical trials."

Highlights at the American Society of Hematology meeting will include:

- Post-hoc efficacy and safety analyses, including a poster focused on the rate of adverse events, from the Phase 3 trial of Vyxeos™ (daunorubicin and cytarabine) liposome for injection, also known as CPX-351
- An oral presentation from an investigator-initiated Phase 2 trial of *Vyxeos* that analyzed remission rates in a group of newly diagnosed elderly acute myeloid leukemia (AML) patients at high risk of mortality
- A post-hoc analysis of the efficacy and safety of *Vyxeos* in patients with Refractory Anemia with Excess of Blasts in Transformation (RAEB-t) from the Phase 3 trial
- Post-hoc analyses from clinical trials and an expanded access program for Defitelio® (defibrotide sodium)

A full list of Jazz-supported oral and poster presentations covering *Vyxeos*, *Defitelio* and Erwinaze® (asparaginase *Erwinia chrysanthemi*) follows below:

Vyxeos™ (daunorubicin and cytarabine) liposome for injection

Presentation Title	**Author**	**Date / Time / Session/ Presentation Number/ Location**
CPX-351 Exposure-Response Based on Cumulative Dose of Cytarabine and Daunorubicin in Patients with Newly Diagnosed, Treatment-related Acute Myeloid Leukemia (AML) or AML with Myelodysplasia-related Changes	Banerjee et al.	December 9 5:30-7:30 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I Poster Presentation 1360 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Rates of Adverse Events Per Patient-Year in a Randomized, Phase 3 Study of CPX-351 Versus 7+3 in Older Adults with Newly Diagnosed, Treatment-related Acute Myeloid Leukemia (AML) or AML with Myelodysplasia-related Changes	Medeiros et al.	December 9 5:30-7:30 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I Poster Presentation 1366 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Subanalysis of Patients with Secondary Acute Myeloid Leukemia (sAML) with Refractory Anemia with Excess of Blasts in Transformation (RAEB-t) Enrolled in a Phase 3 Study of CPX-351 versus Conventional 7+3 Cytarabine and Daunorubicin	Lin et al.	December 9 5:30-7:30 p.m. Session 637. Myelodysplastic Syndromes—Clinical Studies: Poster I Poster Presentation 1698 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Multivariate Efficacy Analysis of a Randomized, Phase 3 Study of CPX-351 Versus 7+3 in Older Adults with Treatment-related Acute Myeloid Leukemia (AML) or AML with Myelodysplasia-related Changes	Uy et al.	December 10 6:00-8:00 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster II Poster Presentation 2647 Georgia World Congress Center,

		Bldg A, Lvl 1, Hall A2
Efficacy and Safety of CPX-351 Versus 7+3 in Older Adults with Secondary Acute Myeloid Leukemia: Combined Subgroup Analysis of Phase 2 and Phase 3 Studies	Lancet et al.	December 10 6:00-8:00 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster II Poster Presentation 2657 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Cost-effectiveness of CPX-351 versus 7+3 Regimen in the Treatment of Treatment-related Acute Myeloid Leukemia (tAML) or AML with Myelodysplasia-related Changes (MRC)	Kansal et al.	December 11 6:00-8:00 p.m. Session 902. Health Services Research—Malignant Conditions: Poster III Poster Presentation 4674 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
CPX-351 Population Pharmacokinetics in Patients with Hematologic Malignancies	Wang et al.	Abstract 5064 Online only in *Blood*
Budget Impact Analysis of CPX-351 in the Treatment of Patients with Treatment-related Acute Myeloid Leukemia (tAML) or AML with Myelodysplasia-related Changes (MRC) from a US Payer Perspective	Jenson et al.	Abstract 5615 Online only in *Blood*

Additionally, the following Jazz-supported Investigator-Initiated Research presentations focusing on *Vyxeos* will be presented at ASH:

Presentation Title	**Author**	**Date / Time / Session/ Presentation Number/ Location**
Randomized Study of CPX-351 for Medically Less-Fit Adults with Newly Diagnosed Acute Myeloid Leukemia or Other High-Grade Myeloid Neoplasm	Walter et al.	December 9 5:30-7:30 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I Poster Presentation 1346 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Phase II Study of CPX-351 (Cytarabine:Daunorubicin) Liposome Injection in Patients with Newly Diagnosed AML at High Risk for Induction Mortality	Borthakur et al.	December 11 6:15-7:45 p.m. Session 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Novel Therapies for Elderly Patients with AML Oral Presentation 892 Georgia World Congress Center, Bldg B, Lvl 5, Murphy BR 1-2

Defitelio® (defibrotide sodium)

Presentation Title	**Author**	**Date / Time / Session/ Presentation Number/ Location**
Efficacy and Safety of Defibrotide in Pediatric Patients with Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) After Hematopoietic Stem Cell Transplantation (HSCT): Final Results from the Expanded-Access Program	Kernan et al.	December 9 5:30-7:30 p.m. Session 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster I Poster Presentation 1948 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2

Pooled Analysis of Day +100 Survival for Defibrotide-Treated Patients With Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) and Ventilator or Dialysis Dependence Following Hematopoietic Stem Cell Transplantation (HSCT)	Richardson et al.	December 9 5:30-7:30 p.m. Session 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster I Poster Presentation 1946 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Final Analysis of Day +100 Survival by Prior Hematopoietic Stem Cell Transplant Type From an Expanded Access Study of Defibrotide for Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome	Richardson et al.	December 10 6:00-8:00 p.m. Session 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster II Poster Presentation 3224 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Adults Receiving Defibrotide for the Treatment of Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) After Hematopoietic Stem Cell Transplantation (HSCT): Final Results from the Expanded-Access Program	Richardson et al.	December 10 6:00-8:00 p.m. Session 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster II Poster Presentation 3225 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Treatment of Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) Post-Hematopoietic Stem Cell Transplantation (HSCT) in Patients With Neuroblastoma: Final Data From the Defibrotide Expanded-Access Program	Grupp et al.	December 11 6:00-8:00 p.m. Session 731. Clinical Autologous Transplantation: Results: Poster III Poster Presentation 4549 Georgia World Congress Center, Bldg A, Lvl 1, Hall A2
Timing of Defibrotide Initiation in Patients with Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) and Multi-Organ Dysfunction (MOD) After Hematopoietic Stem Cell Transplantation (HSCT): Results from a Retrospective Chart Review	Del Pino et al.	Abstract 5461 Online only in *Blood*
Timing of Diagnosis and Severity of Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) at Diagnosis After Hematopoietic Stem Cell Transplantation: Results from a Retrospective Chart Review	Del Pino et al.	Abstract 5462 Online only in *Blood*
Pooled Analysis of Defibrotide Studies in the Treatment of Veno-occlusive Disease/Sinusoidal Obstruction Syndrome (VOD/SOS) after Hematopoietic Stem Cell Transplantation (HSCT) or Chemotherapy Without HSCT	Richardson et al.	Abstract 5467 Online only in *Blood*

Erwinaze® (asparaginase *Erwinia chrysanthemi*)

Presentation Title	**Author**	**Presentation Number/ Date / Time / Location**
Use of Premedication Prior to Asparaginase (ASNase) Administration in Pediatric Acute Lymphoblastic Leukemia (ALL): Effect on Treatment Completion Rate	Bernard et al.	Abstract 5574 Online only in *Blood*

About Vyxeos™
Vyxeos™ (daunorubicin and cytarabine) is a liposome formulation of a fixed combination of daunorubicin and cytarabine for intravenous infusion. In the U.S., *Vyxeos* received FDA marketing approval on August 3, 2017 for the treatment of adults with newly-diagnosed therapy-related AML (t-AML) or AML with myelodysplasia-related changes (AML-MRC).

Due to different dosage recommendations, *Vyxeos* should not be substituted with other daunorubicin and/or cytarabine- containing products. *Vyxeos* can cause allergic reactions, including anaphylaxis and should not be given to patients who are allergic to *Vyxeos* or any of its ingredients. *Vyxeos* may cause heart-related side effects as well as a severe decrease in blood cells which can result in serious infection or bleeding and possibly lead to death. The most common side effects with *Vyxeos* are 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* before prescribing: http://pp.jazzpharma.com/pi/vyxeos.en.USPI.pdf

About Defitelio®
In the U.S., Defitelio® (defibrotide sodium) received 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).

Defitelio is contraindicated in patients currently taking anticoagulants or fibrinolytics and in patients who are allergic to *Defitelio* or any of its ingredients. *Defitelio* may increase the risk of bleeding and should be withheld or stopped if significant bleeding occurs. Patients should be monitored for allergic reactions, especially if there is a history of previous exposure to *Defitelio*. The most common side effects of *Defitelio* are decreased blood pressure, diarrhea, vomiting, nausea and nose bleeds.

Please see full Prescribing Information for *Defitelio* before prescribing: http://pp.jazzpharma.com/pi/defitelio.en.USPI.pdf

About Erwinaze®
Erwinaze® (asparaginase *Erwinia chrysanthemi*) is currently approved in the U.S. for administration via intramuscular injection or via intravenous infusion in conjunction with chemotherapy. It is indicated as a component of a multi-agent chemotherapeutic regimen for the treatment of patients with acute lymphoblastic leukemia (ALL) who have developed hypersensitivity to E. coli-derived asparaginase. *Erwinaze* is derived from the bacterium Erwinia chrysanthemi and is therefore immunologically distinct from E. coli-derived asparaginase and suitable for patients with hypersensitivity to E. coli-derived treatments.

Erwinaze is contraindicated in patients who have had serious allergic reactions to *Erwinaze*, or had serious swelling of the pancreas, serious blood clots, or serious bleeding with past L-asparaginase treatment. *Erwinaze* should be discontinued if any of the following occur: serious allergic reactions, including a feeling of tightness in the throat, unusual swelling/redness in the throat and/or tongue, or trouble bleeding; or severe inflammation of the pancreas. Glucose intolerance has been reported, which in some cases may be irreversible. If blood clots of bleeding occur, discontinue *Erwinaze* until symptoms resolve. The most common side effects of *Erwinaze* are allergic reactions, too much sugar in the blood, fever, swelling of the pancreas, local reactions (swelling, rash, etc. where the needle entered the skin), vomiting, nausea, blood clots, liver problems, stomach pain/discomfort, and diarrhea.

Please see full Prescribing Information for *Erwinaze* before prescribing: https://www.jazzpharma.com/wp-content/uploads/2016/01/erwinaze-en-PI.pdf

About Jazz Pharmaceuticals plc
Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is an international biopharmaceutical company focused on improving patients' lives by identifying, developing and commercializing meaningful products that address unmet medical needs. The company has a diverse portfolio of products and product candidates with a focus in the areas of sleep and hematology/oncology. In these areas, Jazz Pharmaceuticals markets Xyrem® (sodium oxybate) oral solution, Erwinaze® (asparaginase *Erwinia chrysanthemi*), Defitelio® (defibrotide sodium) and Vyxeos™ (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Erwinase® and Defitelio® (defibrotide) in countries outside the U.S. For country-specific product labels, please visit www.jazzpharma.com/products. For more information about Jazz, please visit www.jazzpharmaceuticals.com.

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