Data at ASH 2020 Annual Meeting Highlights Jazz Pharmaceuticals’ Commitment to Advancing Hematology/Oncology Research

November 5, 2020

18 abstracts spanning Jazz’s hematology/oncology therapeutic area to be presented

Three abstracts selected for oral presentation, including long-term results from a Phase 3 study of Vyxeos in older adults with newly diagnosed, high-risk/secondary acute myeloid leukemia

DUBLIN, Nov. 5, 2020 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that 11 company-sponsored abstracts, in addition to two abstracts from collaboration trials with The University of Texas MD Anderson Cancer Center (MD Anderson), one abstract from a cooperative group trial and four abstracts from investigator-sponsored trials, will be presented at the 62nd American Society of Hematology (ASH) Annual Meeting, which will be held December 5-8, 2020 as a virtual event.

“This year, we submitted and had more Jazz abstracts accepted for presentation at ASH than ever before, further demonstrating our commitment to research and innovation in hematology and oncology,” said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development of Jazz Pharmaceuticals. “There continues to be an urgent need for new therapeutics and regimens for people with blood cancers who have limited treatment options or who have a goal to obtain a transplant, and we remain committed to these patients through our ongoing research.”

Oral presentations selected to be presented at ASH include:

- Long-term outcomes and five-year results from the Phase 3 study of Vyxeos® (daunorubicin and cytarabine), also known as CPX-351, in older adults with newly diagnosed, high-risk/secondary acute myeloid leukemia (AML)
- Analysis from a multinational, observational registry study evaluating treatment duration, VOD/SOS resolution and survival in patients with veno-occlusive disease/sinusoidal obstruction syndrome treated with Defitelio® (defibrotide sodium) following hematopoietic cell transplantation
- Results from a Phase 2 study in collaboration with MD Anderson investigating the safety and efficacy of Vyxeos in combination with venetoclax in patients with relapsed or refractory AML

The ASH abstracts are available online at https://ash.confex.com/ash/2020/webprogram/start.html

A full list of Jazz-sponsored oral and ePoster presentations follows below:

### Vyxeos Oral and ePoster Presentations

<table>
<thead>
<tr>
<th>Presentation Topic</th>
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| 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 (AML): Outcomes by Age Subgroup and Among Responders | Lancet, et al.  | Oral Presentation: Monday, December 7 12:15 p.m.  
Session Title: 615. Acute Myeloid Leukemia: Commercially Available Therapy, Excluding Transplantation: Commercially Available Therapy, Excluding Transplantation II Presentation Number: 635 |
| CPX-351 Population Pharmacokinetics in Pediatric and Adult Patients with Acute Myeloid Leukemia (AML) | Wang, et al.    | ePoster Presentation: Monday, December 7 7:00 a.m. – 3:30 p.m.  
Session Title: 615. Acute Myeloid Leukemia: Commercially Available Therapy, Excluding Transplantation: Poster III Poster Number: 2848 |
| Quality-Adjusted Time without Symptoms of Disease and Toxicity (Q-TWiST) Analysis of CPX-351 versus 7+3 in Older Adults with Newly Diagnosed High-Risk/Secondary Acute Myeloid Leukemia (AML) | Cortes, et al.  | ePoster Presentation: Sunday, December 6 7:00 a.m. – 3:30 p.m.  
Session Title: 615. Acute Myeloid Leukemia: Commercially Available Therapy, Excluding Transplantation: Poster II Poster Number: 1946 |
| CPX-351 Exposure-Response Analyses for Efficacy and Safety in Pediatric Patients with Relapsed or Refractory Acute Myeloid Leukemia (AML) | Wang, et al.    | ePoster Presentation: Sunday, December 6 7:00 a.m. – 3:30 p.m.  
Session Title: 615. Acute Myeloid Leukemia: |
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<tr>
<td>Long-term Outcomes of Allogeneic Hematopoietic Cell Transplantation in Patients Enrolled in CPX-351-301, a Randomized Phase 3 Study of CPX-351 versus 7+3 in Older Adults with Newly Diagnosed, High-Risk and/or Secondary AML</td>
<td>Uy, et al.</td>
<td>ePoster Presentation: Monday, December 7 7:00 a.m. – 3:30 p.m. Session Title: 732. Clinical Allogeneic Transplantation: Results: Poster III Poster Number: 3346</td>
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<tr>
<td>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 and 2016 Using Hospital Episode Statistics®</td>
<td>Legg, et al.</td>
<td>ePoster Presentation: Sunday, December 6 7:00 a.m. – 3:30 p.m. Session Title: 613. Acute Myeloid Leukemia: Clinical Studies: Poster II Poster Number: 1929</td>
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<td>Patient Experiences with Liposomal Daunorubicin and Cytarabine (CPX-351) versus Conventional Induction Regimens: An Analysis of Patient-Reported Outcomes Data from a Prospective Trial</td>
<td>LeBlanc, et al.</td>
<td>ePoster Presentation: Sunday, December 6 7:00 a.m. – 3:30 p.m. Session Title: 613. Acute Myeloid Leukemia: Clinical Studies: Poster II Poster Number: 1929</td>
</tr>
<tr>
<td>V-FAST: A Phase 1b Master Trial to Investigate CPX-351 Combined with Various Targeted Agents in Patients with Previously Untreated Acute Myeloid Leukemia</td>
<td>Lin, et al.</td>
<td>ePoster Presentation: Saturday, December 5 7:00 a.m. – 3:30 p.m. Session Title: 615 Acute Myeloid Leukemia: Commercially Available Therapy, Excluding Transplantation: Poster I Poster Number: 1025</td>
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<tr>
<td>Exploratory Analysis of the Efficacy and Safety of CPX-351 versus 7+3 by European LeukemiaNet (ELN) 2017 Risk Groups in a Phase 3 Study of Older Adults with High-Risk/Secondary Acute Myeloid Leukemia</td>
<td>Prebet, et al.</td>
<td>ePoster Presentation: Monday, December 7 7:00 a.m. – 3:30 p.m. Session Title: 615 Acute Myeloid Leukemia: Commercially Available Therapy, Excluding Transplantation: Poster I Poster Number: 1025</td>
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<tr>
<td>Post-Marketing Observational Study to Assess the Incidence of Infusion-Related Reactions in Adult Patients with Therapy-Related Acute Myeloid Leukemia (AML) or AML with Myelodysplasia-Related Changes Who Were Treated with CPX-351</td>
<td>Jacoby, et al.</td>
<td>Accepted for publication only</td>
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**Defitelio Oral and ePoster Presentations**

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<tr>
<td>Treatment Duration, Symptom Resolution, and Survival in Defibrotide-Treated Patients with Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome after Hematopoietic Cell Transplantation: Analysis of a Multinational, Prospective, Observational Registry Study</td>
<td>Locatelli, et al.</td>
<td>Oral Presentation: Saturday, December 5 10:15 a.m. Session Title: 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities Presentation Number: 138</td>
</tr>
<tr>
<td>Final Primary Results from the DEFIFrance Registry Study: Effectiveness and Safety of Defibrotide in the Treatment of Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome after Hematopoietic Cell Transplantation</td>
<td>Mohty, et al.</td>
<td>ePoster Presentation: Sunday, December 6 7:00 a.m. – 3:30 p.m. Session Title: 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster II Poster Number: 2386</td>
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Additionally, data from the following collaboration and investigator-sponsored trials on Vyxeos and Defitelio will be presented:

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<tr>
<td>Phase II Study of CPX–351 Plus Venetoclax in Patients with Acute Myeloid Leukemia (AML)</td>
<td>Kadia, et al.</td>
<td>Oral Presentation: Saturday, December 5 8:30 a.m. Session Title: 616.</td>
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</table>
**Acute Myeloid Leukemia: Novel Therapy, Excluding Transplantation: Novel Combination Therapies in Treatment of Newly Diagnosed AML**

**Presentation Number:** 28

**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)**

Perez, et al.

**ePoster Presentation:** Saturday, December 5 7:00 a.m. – 3:30 p.m.

**Session Title:** 613. Acute Myeloid Leukemia: Clinical Studies: Poster I

**Poster Number:** 987

**Initial Results of a Phase 1 Dose Escalation Study of CPX-351 for Patients with Int-2 or High risk IPSS Myelodysplastic Syndromes (MDS) and Chronic Myelomonocytic Leukemia (CMML) After Failure to Hypomethylating Agents**

Montalban Bravo, et al.

**Accepted for publication only**

**Higher Dose of CPX-351 is Associated With Prolonged Hematologic Recovery: Results from an Interim Safety Analysis of the Randomized, Phase III AMLSG 30-18 Trial**

Gaidzik, et al.

**ePoster Presentation:** Saturday, December 5 7:00 a.m. – 3:30 p.m.

**Session Title:** 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I

**Poster Number:** 1043

**Outpatient Vyxeos Induction without Planned Admission for Select Patients with Secondary Acute Myeloid Leukemia (sAML) Is Safe and Yields Healthcare Resource Savings**

Keiffer, et al.

**ePoster Presentation:** Sunday, December 6 7:00 a.m. – 3:30 p.m.

**Session Title:** 615. Acute Myeloid Leukemia: Commercially Available Therapy, excluding Transplantation: Poster II

**Poster Number:** 1949

**A Phase I/II Trial of CPX-351 + Palbociclib in Patients with Acute Myeloid Leukemia**

Nazha, et al.

**ePoster Presentation:** Sunday, December 6 7:00 a.m. – 3:30 p.m.

**Session Title:** 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster II

**Poster Number:** 1962

**A Phase II Study of CPX-351 as a Novel Therapeutic Approach for Patients with Myelodysplastic Syndromes (MDS) after Hypomethylating Agent Failure**

Nazha, et al.

**Accepted for publication only**

**Preliminary Results of a Phase II Study to Determine the Safety of Defibrotide in Children and Adolescents with Sickle Cell Disease-Associated Acute Chest Syndrome (IND 127812)**

Milner, et al.

**ePoster Presentation:** Saturday, December 5 7:00 a.m. – 3:30 p.m.

**Session Title:** 114. Hemoglobinopathies, Excluding Thalassemia – Clinical: Poster I

**Poster Number:** 805

**A Pilot Trial of Pre-Transplant Risk Stratification and Prophylactic Defibrotide to Prevent Serious Thrombotic Microangiopathy in High-Risk Pediatric Hematopoietic Stem Cell Transplant Patients**

Higham, et al.

**ePoster Presentation:** Monday, December 7 7:00 a.m. – 3:30 p.m.

**Session Title:** 721. Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster III

**Poster Number:** 3307

**Defibrotide for the Treatment of Endotheliitis Complicating Sars-Cov-2 Infection: Rationale and Ongoing Studies as Part of the International Defacovid Study Group**

Moraleda, et al.

**Accepted for publication only**

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**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.


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.
Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a global biopharmaceutical company dedicated to developing and commercializing life-changing medicines that transform the lives of patients 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 and movement disorders, and in oncology, including hematologic malignancies 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.

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