Jazz Pharmaceuticals to Present New Data at the Annual ASH Meeting

November 7, 2016

Eleven Presentations Evaluating Marketed and Investigational Compounds for Hepatic Veno-Occlusive Disease (VOD) and Acute Myeloid Leukemia (AML)

Presentations Include a Sub-Analysis of Phase 3 Data for Vyxeos (CPX-351), an investigational product for the treatment of AML Patients

DUBLIN, Nov. 7, 2016 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that eleven abstracts, including four oral presentations, supporting the company's hematology and oncology portfolio will be presented at *the* 58th American Society of Hematology (ASH) Annual Meeting and Exposition in San Diego, California, December 3-6, 2016.

"The data presentations at ASH reflect our efforts in advancing our diversified pipeline of programs in hematology and oncology, including rare blood disorders such as acute lymphoblastic leukemia (ALL) and AML, and in complications of hematopoietic stem-cell transplantation (HSCT) such as hepatic VOD," said Karen Smith, M.D., Ph.D., global head of research and development and chief medical officer at Jazz Pharmaceuticals. "Of note, we look forward to sharing a post-hoc sub-analysis of Phase 3 survival data following allogeneic HSCT in older high-risk AML patients that compares CPX-351, also known as Vyxeos, with the standard of care."

The following oral and poster presentations focusing on Defitelio® (defibrotide sodium) injection, Erwinaze® (asparaginase Erwinia chrysanthemi) and CPX-351(cytarabine and daunorubicin liposome injection) will be presented at ASH.

Defitelio Related Oral and Poster Presentations

Presentation Title	Author	Presentation Number / Date / Time / Location
Timing of Initiation of Defibrotide Post-Diagnosis of Hepatic Veno-Occlusive Disease (VOD) / Sinusoidal Obstruction Syndrome (SOS) Post-Hematopoietic Stem Cell Transplantation (HSCT): Exploratory Age-Group Analysis From an Expanded Access Study	Grupp S, et al.	Oral Presentation 66: - December 3; 8:45 AM (PT); Manchester Grand Hyatt San Diego, Grand Hall B - Session 721: Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Post-Transplant Complications
Impact on Outcomes of Baseline Bilirubin in Patients with Hepatic VOD/SOS Receiving Defibrotide Treatment: A Post-Hoc Analysis	Richardson P, et al.	Poster Presentation 2213: - December 3; 5:30-7:30 PM (PT); San Diego Convention Center, Hall GH - Session 721: Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster I
Treatment of Hepatic VOD/SOS Post-HSCT in Patients With Acute Leukemias: A Subgroup Analysis From the Defibrotide Expanded-Access Program	Richardson P, et al.	Poster Presentation 3412: - December 4; 6:00-8:00 PM (PT); San Diego Convention Center, Hall GH - Session 721: Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Poster II

VOD Related Abstract

Abstract Title	Author	Details
Stratification of Allogeneic HSCT Patients by Risk of Developing VOD: A Model for Assigning a Risk Score	Strouse C, et al.	Oral Presentation 983: - December 5; 3:45 PM (PT); Manchester Grand Hyatt San Diego, Grand Hall B Session 721: Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities: Post-Transplant Complications II
Diagnosis of VOD/SOS With or Without Multi-Organ Dysfunction (MOD) After HSCT: Analysis of a Multicenter Chart Review	Doede T, et al.	*Online Only Publication;* *Abstract 5756*

Erwinaze Related Oral and Poster Presentations

Presentation Title	Author	Presentation Date / Time / Location
Population Pharmacokinetic Modeling of Intravenous Asparaginase Erwinia Chrysanthemi: Impact of Varied Infusion Rates on Exposure	Zomorodi K, et al.	Poster Presentation 1631: - December 3; 5:30-7:30 PM (PT), San Diego Convention Center, Hall GH - 614: Acute Lymphoblastic Leukemia: Therapy,

		excluding Transplantation: Poster I

CPX-351 Related Oral and Poster Presentations

Presentation Title	Author	Presentation Date / Time / Location
Analysis of Efficacy by Age for Patients Aged 60–75 With Untreated Secondary Acute Myeloid Leukemia (AML) Treated With CPX-351 Liposome Injection Versus Conventional Cytarabine and Daunorubicin in a Phase III Trial	Medeiros B, et al.	Oral Presentation 902: - December 5; 3:00 PM (PT); Marriot Marquis San Diego Marina, San Diego Ballroom AB - Session: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Clinical Trials of Novel Drugs and Combinations in AML
Survival Following Allogeneic Hematopoietic Cell Transplantation in Older High-Risk Acute Myeloid Leukemia Patients Initially Treated With CPX-351 Liposome Injection Versus Standard Cytarabine and Daunorubicin: Subgroup Analysis of a Large Phase III Trial	Lancet J, et al.	Oral Presentation 906: - December 5; 4:00 PM (PT); Marriot Marquis San Diego Marina, San Diego Ballroom AB - Session: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Clinical Trials of Novel Drugs and Combinations in AML
Enhanced Cytarabine and Daunorubicin Population Pharmacokinetics When Administered as CPX-351: A Novel Liposomal Formulation Not Requiring Dose Reduction for Mild Renal or Hepatic Dysfunction	Nikanjam M, et al.	Poster Presentation 3955: - December 5; 6:00 – 8:00 PM (PT); San Diego Convention Center, Hall GH - Session 604: Molecular Pharmacology and Drug Resistance in Myeloid Diseases: Poster III

AML Related Poster

Presentation Title	Author	Presentation Number / Date / Time / Location
Burden of Acute Myeloid Leukemia (AML) Among Older Newly-Diagnosed Patients	Sacks N, et al.	Poster Presentation 4780: - December 5; 6:00 – 8:00 PM (PT); San Diego Convention Center, Hall GH - Session 904: Outcomes Research—Malignant Conditions: Poster III

Additionally, one Jazz-sponsored Investigator Initiated Research poster presentation focusing on CPX-351 as an investigational agent for the treatment of AML will be presented at ASH.

Presentation Title	Author	Presentation Number / Date / Time / Location
CPX-351 for the Treatment of High-Risk Patients (pts) With Acute Myeloid Leukemia (AML)	Assi, R, et al.	Poster Presentation 4047: - December 5; 6:00 – 8:00 PM (PT); San Diego Convention Center, Hall GH - Session 616: Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster III

Full details of the ASH 2016 annual meeting can be found here (http://www.hematology.org/Annual-Meeting/) and abstracts can be found here (https://ash.confex.com/ash/2016/webprogram/start.html).

About Defitelio[1]

In the U.S., Defitelio® (defibrotide sodium) injection 80mg/mL 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) and is the first and only FDA-approved therapy for patients with this rare, potentially fatal complication.

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. (https://defitelio.com/DefitelioPI.pdf)

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

About VOD

HSCT is an aggressive, potentially curative procedure to treat patients with malignant and non-cancerous hematologic disorders such as leukemia, lymphoma and aplastic anemia, and congenital immunodeficiency and autoimmune disorders.[2] VOD is a rare complication of HSCT, which occurs in approximately 9-14% of HSCT patients.[3,4] Hepatic VOD, also known as SOS, is an early and life-threatening complication affecting the sinusoidal

endothelial cells of the liver, which can typically occur within the first 21 days following HSCT.[4,5] Hepatic VOD progresses to multi-organ dysfunction in approximately 30-50% of cases.[5] VOD with multi-organ dysfunction (MOD) is associated with an overall mortality (death) rate of 84%.[3] MOD is characterized by the presence of renal or pulmonary dysfunction.[6,7] VOD is often characterized by sudden weight gain, hepatomegaly (abnormally enlarged liver), and elevated bilirubin.[6,7]

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.[8] 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 treatments9. Outside of the U.S., Erwinaze is sold under the name Erwinase®. Please consult local labeling for product information specific to your country.

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. (https://www.jazzpharma.com/wp-content/uploads/2016/01/erwinaze-en-PI.pdf)

About Vyxeos (CPX-351)
CPX-351 (cytarabine and daunorubicin liposome injection) is an investigational product being evaluated for the treatment of AML and is a combination of cytarabine and daunorubicin encapsulated within a nano-scale liposome at a 5:1 molar ratio. The proposed trade name, Vyxeos™, is conditionally approved by the FDA and is subject to confirmation upon approval of the NDA. CPX-351 was granted orphan drug status by the FDA and the European Commission for the treatment of acute myeloid leukemia. CPX-351 was granted Breakthrough Therapy Designation for the treatment of adults with therapy-related AML or AML with myelodysplasia-related changes and was also granted Fast Track designation by the FDA for the treatment of older patients with secondary AML. On October 3, 2016 Jazz announced the initiation of a rolling submission of a New Drug Application (NDA) to the FDA, seeking marketing approval of CPX-351 for the treatment of AML.

About Acute Myeloid Leukemia
Acute myeloid leukemia (AML) is a rapidly progressing and life-threatening blood cancer that rises in frequency with age.[16] The American Cancer Society estimates that there will be 19,950 new cases of AML and 10,430 deaths from AML in the U.S. in 2016.[10] In the European Union, the number of new cases is estimated to be 20,100 in 2016.[11]

The median age at diagnosis is 67 and with rising age there is progressive worsening of prognosis.[10,12] Advancing age is associated with increasing risk of specific chromosomal/mutational changes and risk of pre-malignant marrow disorders which give rise to more aggressive and less responsive forms of AML.[13,14] As patients age there is also reduced tolerance for intensive chemotherapy.[15] As a consequence, advances in supportive care, intensive chemotherapy, and bone marrow transplantation have primarily benefitted younger patients with approximately one third of patients 18-60 years of age achieving cure.[13,15] Older patients have not achieved higher rates of cure or improved upon a 5-year survival rate of 10-20% in spite of 40 years of research.[15,16]

About Jazz Pharmaceuticals
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) and Defitelio® (defibrotide sodium) in the U.S. and markets Erwinase® and Defitelio® (defibrotide) in countries outside the U.S. For more information, please visit www.jazzpharmaceuticals.com.

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