Jazz Pharmaceuticals To Present Data From Ongoing Evaluations Of Defibrotide At The 2016 BMT Tandem Meeting

February 18, 2016

Jazz-Sponsored Oral Presentations Include: Results from a Pivotal Phase 3 Trial for the Treatment of VOD with MOD, Sub-Analysis Data from a Phase 3 Pediatric Trial for VOD Prophylaxis, and an Exploratory Post-Hoc Analysis from an Expanded Access Treatment of VOD / SOS Study to Evaluate Timing of Treatment Initiation
10 Jazz-Sponsored Posters Also to Be Presented

DUBLIN, Feb. 18, 2016 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that researchers will present three Jazz-sponsored oral abstracts related to the ongoing evaluation of defibrotide, an investigational medicine being studied in the United States (U.S.) for the treatment of adult and pediatric patients with hepatic veno-occlusive disease (VOD), also known as sinusoidal obstruction syndrome (SOS), with evidence of multi-organ dysfunction (MOD) following hematopoietic stem-cell transplantation (HSCT). Data will be presented at the 2016 BMT (Bone Marrow Transplantation) Tandem Meeting, the combined annual meetings of the American Society of Blood and Marrow Transplantation (ASBMT) and the Center for International Blood and Marrow Transplant Research (CIBMTR), taking place from February 18 to 22 in Honolulu, Hawaii.

The three oral scientific presentations include: 1) Results from the pivotal, historically controlled Phase 3 trial of defibrotide for the treatment of hepatic VOD, also known as SOS, with MOD following HSCT; 2) results from a sub-analysis of data from an open-label, randomized, Phase 3 pediatric trial of defibrotide for the prophylaxis of VOD, including patients with specific VOD / SOS risk factors; and 3) results from an exploratory post-hoc analysis of data from an expanded access treatment of VOD / SOS study to evaluate the timing of defibrotide initiation post-VOD diagnosis and impact on Day +100 survival following HSCT.

"The data presented at this year's BMT Tandem Meeting add to the growing body of scientific evidence regarding the efficacy and safety of defibrotide in the treatment of hepatic VOD with MOD following HSCT," said Karen Smith, M.D., Ph.D., global head of research and development and chief medical officer at Jazz Pharmaceuticals. "As we explore additional potential indications for defibrotide, the more we know, the more we might be able to do to fill unmet needs for patients."

The two Phase 3 pivotal study oral presentations will take place on **Thursday, February 18, 2016, from 4:45 to 5:15 pm Hawaii-Aleutian Standard Time (HAST)** during *Session A: Oral Abstracts – Late Effects, Supportive Care and Quality of Life,* in Exhibit Hall 3 at the Hawaii Convention Center:

- **Presentation 7, Paul G. Richardson, M.D., "Defibrotide for the Treatment of Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome with Multi-Organ Dysfunction: Final Results from a Pivotal, Historically Controlled, Phase 3 Trial."** Dr. Richardson and co-authors will present data from an analysis of Day +100 survival (primary endpoint) and complete response (key secondary endpoint) data from a pivotal Phase 3 study of defibrotide in the treatment of VOD with MOD following HSCT. Dr. Richardson is the lead investigator and director of clinical research at the LeBow Institute for Myeloma Therapeutics and the Jerome Lipper Multiple Myeloma Center at the Dana-Farber Cancer Institute and the RJ Corman Professor of Medicine at Harvard Medical School.
- **Presentation 8, Selim Corbacioglu, M.D., "Defibrotide for Prophylaxis of Hepatic Veno-Occlusive Disease in Pediatric Hematopoietic Stem Cell Transplantation: Sub-analysis Data from an Open-Label, Phase 3, Randomized Trial**." Dr. Corbacioglu, of the University of Regensburg, Germany, will present results from a sub-analysis of data from an open-label, randomized, Phase 3 pediatric trial of defibrotide for the prophylaxis of VOD, including patients with specific VOD / SOS risk factors.

The third oral presentation will take place on **Sunday, February 21 from 4:45 to 5:00 pm HAST** during *Session L: Oral Abstracts – Late Effects, Supportive Care and Quality of Life*, in room #316 ABC at the Hawaii Convention Center**:**

- **Presentation 83, Paul G. Richardson, M.D., "Early Initiation of Defibrotide in Patients with Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome Following Hematopoietic Stem Cell Transplantation Improves Day +100 Survival."** Dr. Richardson will present results from an exploratory post-hoc analysis of data from an expanded access treatment of VOD / SOS study to evaluate the timing of defibrotide initiation post VOD diagnosis and impact on Day +100 survival following HSCT.

The 10 posters will be presented in the poster sessions in Exhibit Hall 3 at the Hawaii Convention Center. The following will be presented on **Thursday, February 18 from 6.45 to 7:45 pm HAST:**

- **Poster 254:** N.A. Kernan, et al., "Defibrotide for the Treatment of Patients with Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome with/without Multi-Organ Dysfunction Following Chemotherapy: Subset Analysis Results from an Ongoing Expanded Access Program."
- **Poster 255:** N.A. Kernan, et al. "Pooled Treatment Analysis of Pediatric Patients with Defibrotide for Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome and Multi-Organ Dysfunction Following Hematopoietic Stem Cell Transplant."

- **Poster 398:** C.C. Dvorak, et al. "Hospital Cost Associated with Veno-Occlusive Disease (VOD) in Patients with Hematopoietic Stem Cell Transplant (HSCT)."
- **Poster 409:** T.P. Quock, et al. "A Model Estimating Indirect Costs of Premature Death Associated with Severe Hepatic Veno-occlusive Disease (sVOD) among Hematopoietic Stem Cell Transplant (HSCT) Patients in the United States (U.S.)."
- **Poster 410:** T.P. Quock, et al. "Costs of Hematopoietic Stem Cell Transplantation and Associated Conditioning Regimens."
- **Poster 421:** C.C. Dvorak, et al. "Incidence of Hepatic Veno-Occlusive Disease (VOD) in Premier Healthcare Data."

The following posters will be presented on **Sunday, February 21 from 6:45 to 7:45 pm HAST**:

- **Poster 446:** S. Arai S, et al. "Efficacy and Safety of Defibrotide in a Subset Analysis of Late-Onset Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome (VOD / SOS) Patients from an Ongoing, Expanded-Access Program."
- **Poster 459:** T. Corn, et al. "Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome: Diagnostic Patterns among United States and European Union Hematologists and Oncologists Managing Patients Receiving Stem Cell Transplantation."
- **Poster 500:** P.L. Martin, et al. "Pooled Dose Response Analysis of Defibrotide in >1600 Patients for the Treatment of Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome."
- **Poster 524:** P.G. Richardson, et al. "Defibrotide for Hepatic Veno-Occlusive Disease/Sinusoidal Obstruction Syndrome with Multi-organ Dysfunction: A Concordance Analysis Between Day +100 Complete Response and Survival."

The BMT Tandem Meeting is one of the largest international forums dedicated specifically to HSCT.

Full details of the 2016 BMT Tandem Meeting can be found at https://bmt.confex.com/tandem/2016/meetingapp.cgi/Home/0.

About VOD

HSCT is a potentially curative procedure to treat patients with malignant and non-cancerous hematologic disorders such as leukemia, lymphoma and aplastic anemia, congenital immunodeficiency and autoimmune disorders.[1] Hepatic VOD, also known as SOS, is a rare, early and life-threatening complication of HSCT.[2]

About Defibrotide

In the U.S., defibrotide is an investigational drug for the treatment of patients with hepatic VOD with MOD following HSCT. Defibrotide was granted Orphan Drug Designation by the FDA in May 2003 and has Fast Track designation. A new drug application (NDA) is under review by the U.S. Food and Drug Administration (FDA).

Defibrotide is being made available as an investigational new drug (IND) free of charge through an expanded access Treatment Protocol. The ongoing expanded access Treatment Protocol is currently enrolling patients diagnosed with VOD in the U.S. Expanded access programs are part of an effort by the FDA and the pharmaceutical industry to make investigational drugs available for the treatment of serious or life-threatening diseases in people with limited treatment options. For information about the expanded access defibrotide study, contact Erin Tokunaga at erin.tokunaga@jazzpharma.com or Lam Calderon (1.312.706.6240; 0265-002Gentium@iconplc.com); or visit www.clinicaltrials.gov (Identifier: NCT00628498).

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 hepatic VOD in patients undergoing HSCT therapy. Defitelio received an approval in Israel in May 2015 for the same indication as in the EU.

▼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 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 and Erwinaze® (asparaginase *Erwinia chrysanthemi*) in the U.S., and markets Erwinase® and Defitelio® (defibrotide) in countries outside the U.S. For more information, please visit www.jazzpharmaceuticals.com.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995

This press release contains forward-looking statements, including, but not limited to, statements related to Jazz Pharmaceuticals' development activities with respect to defibrotide and other statements that are not historical facts. These forward-looking statements are based on the company's 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, among others, risks and uncertainties associated with the difficulty and uncertainty of pharmaceutical product development and the uncertainty of clinical success; the uncertainty associated with the regulatory approval process; the company's ability to effectively commercialize its product candidates; and other risks and uncertainties affecting the company, 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 the company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 and future filings and reports by the company. Other risks and uncertainties of which the company is not currently aware may also affect the company's forward-looking statements and may cause actual results and 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 the company on its website or otherwise. The company 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.

References

[1]Ikehara S. New strategies for BMT and organ transplantation. *Int J Hematol*. 2002;76(Suppl 1):161-4.

[2]Coppell JA, Richardson PG, Soiffer R, et al. Hepatic veno-occlusive disease following stem cell transplantation: incidence, clinical course, and outcome. *Biol Blood Marrow Transplant*. 2010;16(2):157-168.

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SOURCE Jazz Pharmaceuticals plc

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