Jazz Pharmaceuticals To Present New Analysis Of Defibrotide Data In Patients With Hepatic Veno-Occlusive Disease (VOD) At ASH Annual Meeting

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Posters Include an Epidemiological Measure of the Effectiveness Analysis of Defibrotide from a Phase 3 Trial in Severe Hepatic VOD, as well as Updates from an International Compassionate Use Program and a U.S. Treatment IND Study

DUBLIN, Dec. 2, 2014 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that researchers will present the results of a number needed to treat (NNT; an epidemiological measure of effectiveness) analysis from a historically controlled Phase 3 clinical trial evaluating the use of defibrotide for the treatment of severe hepatic veno-occlusive disease (severe VOD or sVOD) in patients undergoing hematopoietic stem-cell transplantation (HSCT) therapy. Researchers will also present updates from an international compassionate use program and an expanded access / treatment investigational new drug (IND) study in the United States (U.S.). These two updates will include data evaluating the use of defibrotide in the treatment of VOD, a rare, early complication in patients undergoing HSCT. These data will be presented in posters at the American Society of Hematology (ASH) 56th Annual Meeting and Exposition in San Francisco, California, taking place from December 6 - 9, 2014.

Earlier this year, Jazz Pharmaceuticals acquired the rights to defibrotide in the U.S. and other markets in North America, South America and Central America. Defibrotide has a fast track regulatory path designation in the U.S. The company plans to initiate a rolling new drug application (NDA) submission to the U.S. Food and Drug Administration for defibrotide for the treatment of severe VOD by the end of the year and anticipates completing the submission in the first half of 2015.

In Europe, defibrotide is marketed under the name Defitelio®▼ (defibrotide). Defitelio is the first and only licensed product in Europe for the treatment of severe VOD in patients over one month of age undergoing HSCT therapy.

"The analyses being presented at ASH this year will further enhance our understanding of defibrotide's activity in the treatment of VOD overall, and of severe VOD in particular," said Paul G. Richardson, M.D., clinical program leader 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. "Importantly, the NNT analysis to be presented will provide additional information on the clinical impact of treatment, further supporting the use of defibrotide as a potential therapeutic option for patients in the treatment of severe VOD."

All posters will be presented in the North Building, Hall E of the Moscone Center in San Francisco, and include:

- Saturday, December 6, 2014, 5:30-7:30 PM (PST), Session 721, Poster I: Corbacioglu S, et al., *Defibrotide for the Treatment of Hepatic Veno-Occlusive Disease: An Update from the International Compassionate Use Program in 710 Patients.* Abstract #1138.
- Sunday, December 7, 2014, 6:00-8:00 PM (PST), Session 721, Poster II: Richardson PG, et al., *Defibrotide for the Treatment of Severe Hepatic Veno-Occlusive Disease: An Analysis of Clinical Benefit as Determined by Number Needed to Treat (NNT) to Achieve Complete Response and to Improve Survival.* Abstract #2469.
- Sunday, December 7, 2014, 6:00-8:00 PM (PST), Session 721, Poster II: Richardson PG, et al., *Updated Results from a Large, Ongoing, Treatment IND Study Using Defibrotide for Patients with Hepatic Veno-Occlusive Disease*. Abstract #2470.

In addition, results from an analysis supported by Jazz Pharmaceuticals that modeled the impact of using pediatric-inspired treatment protocols compared with hyper-CVAD protocols on outcomes for adolescents and young adults with Philadelphia negative acute lymphoblastic leukemia (ALL) will be presented in a poster session on Saturday, December 6, 2014, from 5:30-7:30 PM (Abstract #1281).

"We are pleased to be able to contribute to the scientific presentations at this year's ASH meeting and to provide physicians with additional information regarding the treatment of patients with severe VOD and ALL," said Jeffrey Tobias, M.D., executive vice president and chief medical officer of Jazz Pharmaceuticals.

Full details of the ASH annual meeting can be found at https://ash.confex.com/ash/2014/webprogram/start.html.

About Defibrotide

Defibrotide is marketed under the name Defitelio®▼(defibrotide) in Europe. In October 2013, the European Commission granted marketing authorization under exceptional circumstances for Defitelio for the treatment of severe hepatic VOD in patients undergoing HSCT therapy. It is indicated in patients over one month of age. Defitelio is not indicated in patients with hypersensitivity to defibrotide or any of its excipients or with concomitant use of thrombolytic therapy.

Please consult the Defitelio Summary of Product Characteristics (SmPC) **http://www.ema.europa.eu/ema/index.jsp?curl=/pages/medicines/human/medicines/002393/human_med_001646.jsp** before prescribing, particularly in relation to use of medicinal products that increase the risk of hemorrhage, concomitant systemic anticoagulant therapy, medicinal products that affect platelet aggregation, use in patients who have or develop clinically significant acute bleeding requiring blood transfusion, and patients who have hemodynamic instability.

The most frequent adverse events observed during pre-marketing use were hemorrhage, hypotension and coagulopathy.[7] Please consult the Defitelio SmPC for the full list of all side effects reported with Defitelio.

▼ 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.

About VOD

VOD is an early complication in patients undergoing HSCT therapy. In its severe form, VOD can be life-threatening and is associated with multi-organ failure; it is fatal in over 80% of patients.[1,2] HSCTs are performed with curative intent in patients with hematological malignancies, selected solid tumors and some non-malignant disorders, such as serious hemoglobinopathies.[3,4] In the EU, VOD is designated as a rare disease, affecting less than five in 10,000 people. Studies have reported a wide range of incidence rates for VOD. Generally, data indicate that approximately 14% of patients undergoing HSCT develop VOD.[1,5,6]

About Jazz Pharmaceuticals plc

Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a specialty biopharmaceutical company focused on improving patients' lives by identifying, developing and commercializing differentiated products that address unmet medical needs. The company has a diverse portfolio of products and/or product candidates in the areas of sleep, hematology/oncology, pain and psychiatry. The company's U.S. marketed products in these areas include: Xyrem® (sodium oxybate) oral solution, Erwinaze® (asparaginase Erwinia chrysanthemi), Prialt® (ziconotide) intrathecal infusion, Versacloz® (clozapine) oral suspension, FazaClo® (clozapine, USP) HD and FazaClo LD. Jazz Pharmaceuticals also has a number of products marketed outside the United States, including Erwinase® and Defitelio®▼ (defibrotide). 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' plans to initiate and complete a rolling NDA submission for defibrotide in the U.S. and the timing thereof and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' current expectations 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, without limitation, risks and uncertainties associated with the inherent uncertainty associated with the regulatory approval process, including the risks that the company may be required to conduct additional time-consuming and costly clinical trials prior to submission of the NDA or as a condition of regulatory approval of defibrotide in the U.S. and that the company may otherwise be unable to obtain or maintain any regulatory approval for defibrotide in the U.S., and those other risks detailed 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 and future filings and reports by the company. Jazz Pharmaceuticals undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

References

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7. Defitelio® Summary of product characteristics, 2013.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/jazz-pharmaceuticals-to-present-new-analysis-of-defibrotide-data-in-patients-with-hepatic-veno-occlusive-disease-vod-at-ash-annual-meeting-300003674.html

SOURCE Jazz Pharmaceuticals plc

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