



## **JAZZ PHARMACEUTICALS ANNOUNCES AGREEMENT TO ACQUIRE RIGHTS TO DEFIBROTIDE IN THE AMERICAS FROM SIGMA-TAU PHARMACEUTICALS, INC.**

*Jazz Pharmaceuticals to own worldwide rights to defibrotide at closing*

*Investor conference call to be held today, July 2 at 8:30 AM EDT/1:30 PM IST*

DUBLIN, July 2, 2014 -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the company has signed a definitive agreement with Sigma-Tau Pharmaceuticals, Inc. (Sigma-Tau) under which a subsidiary of Jazz Pharmaceuticals plc (Jazz) will acquire from Sigma-Tau rights to defibrotide in the United States (U.S.) and all other countries in the Americas. Sigma-Tau holds rights to market defibrotide in the Americas under an agreement with Gentium S.p.A., which was acquired by Jazz earlier this year. Defibrotide is a novel product that is marketed by Jazz in the European Union (EU) under the name Defitelio® for the treatment of severe hepatic veno-occlusive disease (VOD) in patients over one month of age undergoing hematopoietic stem cell transplantation (HSCT) therapy. In the U.S., Jazz is working with the Food and Drug Administration (FDA) on the regulatory pathway for submission of a New Drug Application (NDA) for the potential approval of defibrotide to treat patients with severe VOD.

As part of the agreement, Sigma-Tau would receive an upfront payment of \$75 million upon closing of the transaction. Sigma-Tau would be eligible to receive milestone payments of \$25 million upon the acceptance for filing by the FDA of the first NDA for defibrotide for VOD and up to an additional \$150 million based on the timing of potential FDA approval of defibrotide for VOD. Jazz expects to fund the transaction with cash on hand and that the transaction will close during the third quarter of 2014, subject to customary closing conditions, including expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

“The acquisition of the remaining worldwide rights to defibrotide is a strong strategic fit with our specialty focus and would continue the momentum of our recent launch of Defitelio in the EU, further leveraging our global clinical and commercial expertise in hematology/oncology,” said Bruce Cozadd, chairman and chief executive officer of Jazz Pharmaceuticals plc. “This transaction supports our mission to improve care for patients with serious medical conditions, and we remain committed to making targeted investments to develop and bring to market differentiated treatments for patients.”

“This transaction would not only strengthen our global presence, but also demonstrate our commitment to diversify and expand our U.S. commercial portfolio with meaningful new therapies,” said Russell Cox, executive vice president and chief operating officer of Jazz Pharmaceuticals plc. “We look forward to ongoing discussions with the FDA as we continue our efforts toward submission of an NDA for defibrotide in the U.S. Patients in the U.S. with severe VOD have a critical unmet medical need, and we believe that defibrotide has the potential to become an important treatment option for these patients.”

### **About Defitelio®<sup>▼</sup> (defibrotide)**

In October 2013, the European Commission granted marketing authorization under exceptional circumstances for Defitelio®<sup>▼</sup> (defibrotide) for the treatment of severe VOD in 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.

In addition to its existing approved indication in the EU, defibrotide has the potential to be developed for approval in countries outside the EU and in other indications. Defibrotide has been granted orphan drug designation to treat and prevent VOD by the FDA, by the European Medicines Agency (EMA) and by the Korean Ministry of Food and Drug Safety (MFDS), orphan drug designation for the treatment of VOD by the Commonwealth of Australia-Department of Health, and Fast Track designation to treat severe VOD by the FDA.

In the EU, please consult the Defitelio summary of product characteristics 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.

▼ This medicinal product is subject to additional monitoring.

### **About VOD**

Hepatic veno-occlusive disease (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 and is fatal in over 80% of patients.<sup>1,2</sup> HSCTs are performed with curative intent in patients with hematological malignancies, selected solid tumors and some non-malignant disorders, such as serious hemoglobinopathies.<sup>3,4</sup> Studies have reported a wide range of incidence rates for VOD, suggesting that 5-15% of patients undergoing HSCT develop VOD.<sup>2,5,6</sup>

### **Conference Call Information**

Jazz Pharmaceuticals will host a conference call and live audio webcast today at 8:30 a.m. EDT/1:30 p.m. IST to discuss this transaction and related matters. Interested parties may access the live audio webcast and slide presentation via the Investors & Media section of the Jazz Pharmaceuticals website at [www.jazzpharmaceuticals.com](http://www.jazzpharmaceuticals.com). Please connect to the website prior to the start of the conference call to ensure adequate time for any software downloads that may be necessary to listen to the webcast. A replay of the webcast will be archived on the website for one week.

### **Audio Webcast/Conference Call:**

U.S. Dial-In Number: +1 866 543 6403

Outside the U.S. Dial-In Number: +1 617 213 8896

Passcode: 11784518

A replay of the conference call will be available through July 9, 2014 and accessible through one of the following telephone numbers and entering the passcode:

Replay U.S. Dial-In Number: +1 888 286 8010

Replay Outside the U.S. Dial-In Number: +1 617 801 6888

Passcode: 47229125

### **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 U.S., including Erwinaze® and Defitelio® (defibrotide). For more information, please visit [www.jazzpharmaceuticals.com](http://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 the anticipated closing of the acquisition of rights to defibrotide in the U.S. and all other countries in the Americas and the timing and benefits thereof, the therapeutic and commercial potential of defibrotide, planned future discussions with the FDA concerning the regulatory pathway for submission of an NDA for defibrotide, the potential acceptance for filing of and potential approval of an NDA for defibrotide, potential future development of defibrotide for approval in countries outside the EU and in other indications, the company's pipeline and portfolio growth strategy, including potentially bringing new therapies to market, 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 company's ability to complete the acquisition of defibrotide rights from Sigma-Tau on the proposed terms and schedule, including risks and uncertainties related to the satisfaction of closing conditions; the company's ability to successfully manage the risks associated with integrating defibrotide into the company's product portfolio; the possibility that Jazz Pharmaceuticals may fail to realize the anticipated benefits (commercial or otherwise) from the acquisition of defibrotide rights from Sigma-Tau; the inherent uncertainty associated with the regulatory approval process, including the risks that Jazz Pharmaceuticals may be required to conduct additional time-consuming and costly clinical trials in order to obtain any regulatory approval of defibrotide in the U.S. and that Jazz Pharmaceuticals may otherwise be unable to obtain or maintain any regulatory approvals for defibrotide in the U.S. or in other countries outside of the EU; the difficulty and uncertainty of pharmaceutical product development, including the timing and cost thereof; risks related to effectively commercializing defibrotide, including uncertainty of the future sales of and revenue from defibrotide following regulatory approval, if any, in the U.S., and in other indications; the company's ability to identify and acquire, in-license or develop additional products or product candidates to grow its business; possible restrictions on the company's ability and flexibility to pursue certain future opportunities as a result of its substantial outstanding debt obligations; other risks related to future opportunities and plans; 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 March 31, 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:**

1. Carreras E. Chapter 11: Early complications after HSCT. EBMT-ESH Handbook 2012
2. Coppel JA et al. Biol Blood Marrow Transplant. 2010;16:157–168
3. Tsakiris DA & Tichelli A. Best Pract Res Clin Haematol. 2009;22:137–145

4. Majhail NS et al. Bone Marrow Transplant. 2013;48:294–300
5. Carreras E et al. Blood. 1998;92(10):3599-604
6. Corbaciouglu S et al. Lancet. 2012;379(9823):131-9.

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