Jazz Pharmaceuticals Announces First Patient Enrolled in Phase 3 Clinical Study Evaluating JZP-258 for the Treatment of Cataplexy and Excessive Daytime Sleepiness in Narcolepsy

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DUBLIN, March 15, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the first patient has been enrolled in a Phase 3 clinical study evaluating JZP-258, an investigational oxybate product candidate with 90 percent less sodium content than Xyrem® (sodium oxybate) oral solution, as a potential treatment for cataplexy and excessive daytime sleepiness (EDS) in adult narcolepsy patients. The clinical study will be conducted across approximately 60 centers in the European Union and the United States.

"We believe that reducing sodium intake in narcolepsy patients is a clinically meaningful goal as patients with narcolepsy are at risk for high sodium intake-related consequences, including hypertension and other cardiovascular diseases," said Karen Smith, M.D., Ph.D., global head of research and development and chief medical officer of Jazz Pharmaceuticals. "The initiation of patient enrollment in this study reinforces our commitment to the narcolepsy community and is an important step in our efforts to provide patients with potentially improved therapeutic options to treat their narcolepsy."

The Phase 3 study is a double-blind, placebo-controlled, randomized-withdrawal, multicenter study evaluating the efficacy and safety of JZP-258 for the treatment of cataplexy and EDS in adult patients with narcolepsy (ClinicalTrials.gov identifier: NCT03030599). The study is expected to enroll approximately 185 patients in order to randomize approximately 130 patients.

About JZP-258

JZP-258 is an investigational product candidate being evaluated in adult patients for the treatment of cataplexy and EDS in narcolepsy. JZP-258 was evaluated in a Phase 1 clinical study in 60 healthy adults and exhibited a safety profile similar to Xyrem.[1] Xyrem is currently approved for narcolepsy with cataplexy and EDS and, at the highest approved dose of 9 grams per night, contains 1,640 mg of sodium. JZP-258 is an oral solution that contains a mixture of oxybate salts, resulting in 90% less sodium content than Xyrem.

About Narcolepsy

Narcolepsy is a chronic sleep disorder that involves the brain's inability to regulate sleep-wake cycles normally. It affects an estimated 1 in 2,000 people in the United States, with symptoms typically appearing in early adulthood. It is estimated that 50 percent or more patients with narcolepsy have not been diagnosed. Studies have shown it may take 10 years or more for people with narcolepsy to receive a correct diagnosis. Beyond the challenges with narcolepsy diagnosis, scientific publications report that individuals with narcolepsy have an increased frequency of multiple organic diseases, including heart disease, hypertension, and hypercholesterolemia compared to matched controls in the general population.[2,3]

Dietary Sodium Intake Recommendations

High consumption of sodium in diets has also been strongly linked to the development of high blood pressure and the risk of heart disease.[4] The American Heart Association recommends that an ideal diet be limited to no more than 1,500 mg of sodium per day for most adults.[5]

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.

"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 JZP-258 as a potential treatment for cataplexy and excessive daytime sleepiness (EDS) in adult patients with narcolepsy 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, without limitation, risks and uncertainties associated with: pharmaceutical product development and clinical success thereof; and the regulatory approval process; and other risks and uncertainties affecting the company and its development programs, 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 Annual Report on Form 10-K for the year ended December 31, 2016 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 the 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. Data from Phase 1 clinical trial on file.

2. Ohayon MM. Narcolepsy is complicated by high medical and psychiatric comorbidities: a comparison with the general population. Sleep Med. 2013;14:488-492.

3. Jennum P, Ibsen R, Knudsen S, Kjellberg J. Comorbidity and mortality of narcolepsy: a controlled retro- and prospective national study. Sleep. 2013;36:835-840.

4. IOM (2013). "Sodium Intake in Populations: Assessment of Evidence," Washington DC. The National Academies Press.

5. American Heart Association link downloaded March14, 2017. http://www.heart.org/HEARTORG/HealthyLiving/HealthyEating/Nutrition/Sodium-and-Salt_UCM_303290_Article.jsp#.WMLYem8rLIU

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