Jazz Pharmaceuticals Announces First Patients Enrolled in Phase 3 Trial of Xyrem® (Sodium Oxybate) In Children And Adolescents Who Have Narcolepsy With Cataplexy

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Trial Initiated in Response to a FDA Pediatric Written Request to Study Xyrem in Children and Adolescents

DUBLIN, Dec. 2, 2014 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the first patients have been enrolled in a Phase 3 clinical trial to assess the safety and efficacy of Xyrem® (sodium oxybate) in children and adolescents aged seven to 17 who have narcolepsy with cataplexy.

Xyrem is the only U.S. Food and Drug Administration (FDA) approved treatment for narcolepsy with cataplexy in adults. The FDA approval was based on clinical data in primarily adult patients. While there has been a great deal of interest from the narcolepsy community to understand the utility of Xyrem in children or adolescents, there are no published randomized, placebo-controlled trials of Xyrem in pediatric patients. Given the limited knowledge about the use of Xyrem in children or adolescents, Jazz Pharmaceuticals worked with the FDA and key thought leaders to determine the viability of a clinical study to assess Xyrem in children and adolescents. Based on this work, the FDA submitted a *Pediatric Written Request* to Jazz Pharmaceuticals.

"Narcolepsy with cataplexy is a debilitating, chronic condition that commonly begins in childhood, yet there are no approved cataplexy treatments for patients under the age of 18," said Jed Black, M.D., vice president, Sleep Medicine, Jazz Pharmaceuticals, and consulting associate professor, Stanford University Medical Center, Stanford Center for Sleep Sciences and Medicine. "Jazz Pharmaceuticals is pleased to conduct this study with the hope that it will provide the narcolepsy community, the FDA and physicians more information about the use of Xyrem in pediatric patients who currently have limited treatment options."

The Xyrem Pediatric Narcolepsy Study is a 52-week, Phase 3 randomized, double-blind, open-label, multicenter clinical trial that will evaluate the safety, efficacy and pharmacokinetics of Xyrem in patients aged seven to 17. The trial will enroll up to 100 pediatric patients globally at sites in the U.S. and several countries in Europe.

Additional information about the trial, including eligibility criteria and a list of clinical trial sites can be found at: www.clinicaltrials.gov (ClinicalTrials.gov Identifier: NCT02221869)

About Xyrem

Xyrem® (sodium oxybate) oral solution, CIII, is indicated for the treatment of cataplexy in narcolepsy and for the treatment of excessive daytime sleepiness (EDS) in narcolepsy. Xyrem may only be dispensed to patients enrolled in the Xyrem Success Program. Xyrem was first approved in the U.S. in 2002. Safety and effectiveness in pediatric patients have not been established.

IMPORTANT SAFETY INFORMATION

XYREM is a Central Nervous System (CNS) depressant. In clinical trials at recommended doses, obtundation and clinically significant respiratory depression occurred in XYREM-treated patients. Almost all of the patients who received XYREM during clinical trials in narcolepsy were receiving CNS stimulants.

XYREM is the sodium salt of gamma hydroxybutyrate (GHB). Abuse of GHB, either alone or in combination with other CNS depressants, is associated with CNS adverse reactions, including seizure, respiratory depression, decreases in the level of consciousness, coma, and death.

Because of the risks of CNS depression, abuse, and misuse, XYREM is available only through a restricted distribution program called the XYREM Success Program®, using a centralized pharmacy. Prescribers and patients must enroll in the program. For further information go to www.XYREM.com or call 1-866-XYREM88® (1-866-997-3688).

Xyrem is contraindicated in combination with sedative hypnotics or alcohol and in patients with succinic semialdehyde dehydrogenase deficiency. Use caution when considering the concurrent use of Xyrem with other CNS depressants. Healthcare providers should caution patients against hazardous activities requiring complete mental alertness or motor coordination within the first 6 hours of dosing or after first initiating treatment until certain that Xyrem does not affect them adversely. Xyrem is a Schedule III controlled substance. The rapid onset of sedation, coupled with the amnestic features of Xyrem, particularly when combined with alcohol, has proven to be dangerous for the voluntary and involuntary user (e.g. assault victim). Monitor patients for emergent or increased depression and suicidality and for impaired motor/cognitive function. Episodes of sleepwalking should be fully evaluated and appropriate interventions considered. Consider the amount of daily sodium intake in each dose of Xyrem in patients sensitive to salt intake.

In three controlled clinical trials, the most common adverse reactions (incidence ≥5% and twice the rate of placebo) in Xyrem-treated patients were nausea (20%), dizziness (15%), vomiting (11%), somnolence (8%), enuresis (7%) and tremor (5%).

Please click here to see the full Prescribing Information for Xyrem, including BOXED Warning (http://www.xyrem.com/images/XYREM_PI.pdf)

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' ongoing clinical trial to assess the safety and efficacy of Xyrem in children and adolescents and the results 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 timing, scope and results of the clinical trial and those other risks with respect to research and development and clinical trials 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.

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SOURCE Jazz Pharmaceuticals plc

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