Jazz Pharmaceuticals Announces First Patient Enrolled in Phase 3 Clinical Trial Evaluating JZP-258 for the Treatment of Idiopathic Hypersomnia

November 29, 2018

DUBLIN, Nov. 29, 2018 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the first patient has been enrolled in a Phase 3 clinical trial evaluating the efficacy and safety of JZP-258 for the treatment of idiopathic hypersomnia, a sleep disorder characterized by chronic and disabling excessive daytime sleepiness (daytime periods of irrepressible need to sleep or daytime lapses into sleep) that is not caused by other conditions known to induce excessive daytime sleepiness. Other symptoms of idiopathic hypersomnia may include prolonged nighttime sleep, long and unrefreshing naps and difficulty waking up from nocturnal sleep or daytime naps. JZP-258 is an investigational oxybate mixed-salts oral solution with 90 percent less sodium than *Xyrem*® (sodium oxybate) oral solution. JZP-258 is also currently in Phase 3 development for the treatment of excessive daytime sleepiness and cataplexy in narcolepsy.

The JZP-258 clinical trial for patients with idiopathic hypersomnia will be conducted in multiple study centers in the United States (U.S.) and European Union (EU).

"Idiopathic hypersomnia is a debilitating orphan disease and an area of significant unmet patient need since there are no therapies approved to treat it and general awareness of idiopathic hypersomnia is low," said Jed Black, M.D., senior vice president, Sleep and CNS Medicine at Jazz Pharmaceuticals and adjunct professor, Stanford Center for Sleep Sciences and Medicine. "This clinical trial is an example of Jazz's commitment to collaborating with the sleep community to advance sleep science and develop potential new treatment options for people with chronic, disabling sleep disorders."

The Phase 3 clinical trial is a double-blind, placebo-controlled, randomized-withdrawal, multicenter study evaluating the efficacy and safety of JZP-258 for the treatment of idiopathic hypersomnia, with an open-label safety extension. Jazz expects to enroll approximately 140 adult patients with idiopathic hypersomnia. The primary endpoint is change in Epworth Sleepiness Scale (ESS) score. Secondary endpoints include the Patient Global Impression of Change (PGIc), the Clinical Global Impression of Change (CGIc), and change in total score on the Hypersomnolence Severity Scale (HSS).

Additional information about the trial, including eligibility criteria and a list of clinical trial sites, can be found at: https://clinicaltrials.gov (ClinicalTrials.gov Identifier: NCT03533114).

About JZP-258

JZP-258 is an investigational product candidate being evaluated in adult patients for the treatment of cataplexy and excessive daytime sleepiness in narcolepsy and for the treatment of idiopathic hypersomnia. JZP-258 is an oral solution that contains a mixture of oxybate salts, resulting in 90 percent less sodium content than Xyrem® oral solution.

About Idiopathic Hypersomnia

Idiopathic hypersomnia is a sleep disorder characterized by chronic and disabling excessive daytime sleepiness (daytime periods of irrepressible need to sleep or lapse into sleep) that is not caused by other medical, behavioral or psychiatric conditions known to induce excessive sleepiness.[1,2,3,4] Symptoms may include prolonged nighttime sleep, long and unrefreshing naps and difficulty waking up from nocturnal sleep or daytime naps.[1,2,3,4] Idiopathic hypersomnia is a debilitating illness that can significantly affect social, school and occupational functioning.

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 therapeutic areas, Jazz Pharmaceuticals markets Xyrem® (sodium oxybate) oral solution, Erwinaze® (asparaginase Erwinia chrysanthemi), Defitelio® (defibrotide sodium) and Vyxeos® (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Erwinase®, Defitelio® (defibrotide) and Vyxeos® 44 mg/100 mg powder for concentrate for solution for infusion in countries outside the U.S. For country-specific product information, please visit http://www.jazzpharmaceuticals.com/products. For more information, please visit https://www.jazzpharmaceuticals.com/ and follow us on Twitter at @JazzPharma.

"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 idiopathic hypersomnia, Jazz Pharmaceuticals' plans and expectations regarding its Phase 3 clinical trial evaluating JZP-258 for the treatment of idiopathic hypersomnia, 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, including risks related to failure or delays in completing clinical trials; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 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. 2015 Review article in Chest, by Khan/Trotti et al, "Central Disorders of Hypersomnolence: Focus on the Narcolepsies and Idiopathic Hypersomnia" https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4694150/
2. 2016 Sleep Medicine Review article, by Billiard/Sonka et al, "Idiopathic Hypersomnia " https://www.ncbi.nlm.nih.gov/pubmed/26599679
3. International Classification of Sleep Disorders, Third Edition (ICSD-3): http://www.aasmnet.org/store/product.aspx?pid=849
4. Diagnostic and Statistical Manual of Mental Disorders (DSM-V) p. 368-372 Hypersomnolence Disorder: https://www.psychiatry.org/psychiatrists/practice/dsm

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SOURCE Jazz Pharmaceuticals plc

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