Jazz Pharmaceuticals Receives Positive CHMP Opinion for Solriamfetol to Improve Wakefulness and Reduce Excessive Daytime Sleepiness in Adults with Narcolepsy or Obstructive Sleep Apnea

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Solriamfetol is a dual-acting dopamine and norepinephrine reuptake inhibitor shown to improve wakefulness in adults living with excessive daytime sleepiness associated with narcolepsy or obstructive sleep apnea

If approved by the European Commission for this indication, solriamfetol will be the only licensed therapy in Europe for the treatment of excessive daytime sleepiness in adults living with obstructive sleep apnea

DUBLIN, Nov. 15, 2019 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion recommending the marketing authorisation of solriamfetol to improve wakefulness and reduce excessive daytime sleepiness (EDS) in adults with narcolepsy (with or without cataplexy) or obstructive sleep apnea (OSA) whose EDS has not been satisfactorily treated by primary OSA therapy, such as continuous positive airway pressure (CPAP).[1]

"Today's positive CHMP opinion is an important milestone for people living with EDS associated with narcolepsy or OSA, which has been shown to negatively impact the lives of people living with these conditions," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development of Jazz Pharmaceuticals. "This milestone brings us one step closer to potentially providing a new treatment option for people living with these sleep disorders in Europe."

The Marketing Authorisation Application (MAA) for solriamfetol is based on data from four randomised placebo-controlled studies included in the Treatment of Obstructive sleep apnea and Narcolepsy Excessive Sleepiness (TONES) clinical trial program. Data from the studies in the TONES program demonstrated the superiority of solriamfetol relative to placebo.[2-5]

The CHMP recommended that once daily solriamfetol be approved with doses of 75 mg and 150 mg for people with narcolepsy and doses of 37.5 mg, 75 mg and 150 mg for people with OSA.[1]

"Excessive daytime sleepiness due to narcolepsy or OSA may have negative impacts on a person's ability to function at work or at home, and in OSA patients EDS can still occur despite the compliant use of CPAP treatment for upper airway obstruction," said Professor Jean-Louis Pépin, M.D., Ph.D., Director of INSERM unit 1042 and head of the sleep and physiology department at the University Hospital in Grenoble, France. "I am hopeful this positive CHMP opinion leads to this novel treatment option becoming available for people living with EDS as a result of narcolepsy or OSA."

Jazz Pharmaceuticals filed the MAA for solriamfetol with the EMA in November 2018. The positive opinion from the CHMP will now be reviewed by the European Commission (EC), which has the authority to approve medicines in all European Union Member States, Iceland, Norway and Liechtenstein. A decision from the EC is expected within 67 days of receiving the CHMP opinion.

About solriamfetol

Solriamfetol is a dual-acting dopamine and norepinephrine reuptake inhibitor (DNRI) shown to improve wakefulness in adults living with excessive daytime sleepiness due to narcolepsy or obstructive sleep apnea (OSA). [2-7] Solriamfetol, marketed as Sunosi® in the U.S., received U.S. Food and Drug Administration (FDA) approval on March 20, 2019 to improve wakefulness in adult patients with EDS associated with narcolepsy or OSA and was designated a Schedule IV medicine by the U.S. Drug Enforcement Agency (DEA) on June 17, 2019.[8,9] In 2014, Jazz Pharmaceuticals acquired a license to develop and commercialize solriamfetol from Aerial Biopharma LLC. Jazz Pharmaceuticals has worldwide development, manufacturing, and commercialization rights to solriamfetol, excluding certain jurisdictions in Asia. SK Biopharmaceuticals Co., Ltd., the discoverer of the compound, maintains rights in 12 Asian markets, including Korea, China, and Japan. *Sunosi* has orphan drug designation for narcolepsy in the United States.

About Obstructive Sleep Apnea and Excessive Daytime Sleepiness

Obstructive sleep apnea (OSA), commonly referred to as sleep apnea, is a highly prevalent disease affecting approximately 44% of Europeans.[10] Excessive daytime sleepiness (EDS), a major symptom of OSA, is characterized by the inability to stay awake and alert during the day resulting in unplanned lapses into sleep or drowsiness.[11,12] Positive Airway Pressure (PAP) therapy, with its most common form being Continuous Positive Airway Pressure (CPAP), has been shown to be an effective therapy for sleep apnea that frequently results in improvement in EDS in many patients;[13,14] however, not all patients tolerate CPAP therapy and among those who tolerate CPAP, usage is highly variable.[15,16,17] EDS may persist in people with OSA despite using CPAP.[18,19]

About Narcolepsy

Narcolepsy is a chronic, debilitating neurological disorder characterized by excessive daytime sleepiness (EDS), and the inability to regulate sleep-wake cycles normally.[20] Narcolepsy is a rare disease with an estimated prevalence of 0.02% in European populations.[21] Studies have shown it may take 10 years or more for people with narcolepsy to receive a diagnosis, and it is estimated that more than 50% of patients with narcolepsy have not been diagnosed.[22,23,24] There are five primary symptoms of narcolepsy, including EDS, cataplexy, sleep-related hallucinations, sleep paralysis and sleep disruption.[11] While all patients with narcolepsy experience EDS, they may not experience all five symptoms.[11]

About Jazz Pharmaceuticals

Jazz Pharmaceuticals plc (Nasdaq: JAZZ), a global biopharmaceutical company, is dedicated to developing life-changing medicines for people with limited or no options. As a leader in sleep medicine and with a growing hematology/oncology portfolio, Jazz has a diverse portfolio of products and product candidates in development, and is focused on transforming biopharmaceutical discoveries into novel medicines. Jazz Pharmaceuticals markets Sunosi® (solriamfetol), Xyrem® (sodium oxybate) oral solution, Defitelio® (defibrotide sodium), Erwinaze® (asparaginase *Erwinia chrysanthemi*) and Vyxeos® (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Defitelio® (defibrotide), Erwinase® and

Vyxeos® liposomal 44 mg/100 mg powder for concentrate for solution for infusion in countries outside the U.S. For country-specific product information, please visit https://www.jazzpharmaceuticals.com/medicines. For more information, please visit 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 Jazz Pharmaceuticals' belief that *Sunosi*, if approved in the European Union, will potentially provide a new treatment option for people living with sleep disorders in Europe, Jazz Pharmaceuticals' timing expectation regarding the EC's decision with respect to the *Sunosi* MAA, and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' 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: the uncertain regulatory approval process, including the risk that Jazz Pharmaceuticals' MAA for *Sunosi* may not be approved by the EC in a timely manner or at all; effectively commercializing *Sunosi*, if approved, in Europe; and other risks and uncertainties affecting Jazz Pharmaceuticals 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 Jazz Pharmaceuticals' Quarterly Report on Form 10-Q for the quarter ended September 30, 2019 and future filings and reports by Jazz Pharmaceuticals. Other risks and uncertainties of which Jazz Pharmaceuticals is not currently aware may also affect Jazz Pharmaceuticals' 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 Jazz Pharmaceuticals on its website or otherwise. Jazz Pharmaceuticals 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.

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