



## Jazz Pharmaceuticals to Present Phase 3 Data on JZP-110, an Investigational Treatment for Excessive Sleepiness in Patients with Narcolepsy and with Obstructive Sleep Apnea, During 31st Annual SLEEP Meeting

May 24, 2017

### Twelve Jazz-Sponsored Oral and Poster Presentations To Be Presented

DUBLIN, Ireland, May 24, 2017 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) announced today that twelve abstracts supporting the company's sleep medicine portfolio, including data on its investigational compound JZP-110, data on the investigational use of sodium oxybate for the treatment of cataplexy and excessive daytime sleepiness in pediatric patients with narcolepsy, and data from the Nexus narcolepsy patient registry, will be presented at the 31<sup>st</sup> Associated Professional Sleep Societies (APSS) Annual SLEEP Meeting June 3-7 in Boston, MA.

"Developing innovative treatments for often overlooked and debilitating conditions is one of our highest priorities," said Karen Smith, M.D., Ph.D., executive vice president, research and development and chief medical officer at Jazz Pharmaceuticals. "The breadth of data that we will be presenting at the SLEEP 2017 meeting reflects our ongoing commitment to advancing meaningful treatments that address unmet needs in sleep medicine, including for patients living with narcolepsy and obstructive sleep apnea (OSA)."

Key presentations include data from the Treatment of OSA and Narcolepsy Excessive Sleepiness (TONES) Phase 3 program for JZP-110, an investigational wake-promoting agent in development for the treatment of excessive sleepiness in adult patients with narcolepsy (TONES 2) or OSA (TONES 3 and TONES 4), Phase 2/3 data from a clinical study evaluating sodium oxybate in pediatric patients aged 7-17, and new data from the Jazz-sponsored Nexus Narcolepsy Registry, the first narcolepsy-specific, web-based registry of patient-reported data.

A full list of Jazz-supported oral and poster presentations follows below:

#### JZP-110-related Poster Presentations

| Presentation Title  | Author              | Presentation Date/Time   |
|---|---------------------|--|
| <i>An Open-Label, Single-Dose, Phase 1 Study of the Pharmacokinetics and Safety of JZP-110 in Subjects with Normal or Impaired Renal Function and with End-Stage Renal Disease Requiring Hemodialysis</i>                                       | K. Zomorodi et al   | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Sunday Jun 4, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 1027</li> <li>• Poster Number: 291</li> <li>• Session ID: P11</li> </ul>     |
| <i>Comorbidities and Health-related Quality of Life Among People with Sleep Apnea with Excessive Daytime Sleepiness: Findings from the 2016 US National Health and Wellness Survey</i>  | C. Stepnowsky et al | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Sunday Jun 4, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0457</li> <li>• Poster Number: 41</li> <li>• Session ID: P02</li> </ul>      |
| <i>A Randomized, Placebo-Controlled, Phase 3 Study of the Safety and Efficacy of JZP-110 for the Treatment of Excessive Sleepiness in Patients with Narcolepsy</i>  | M. Thorpy et al     | <b>Poster presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0675</li> <li>• Poster Number: 106</li> <li>• Session ID: P28</li> </ul>    |
| <i>A Phase 3, Randomized, Placebo-Controlled, Double-Blind, 12-Week, Multicenter Study of the Efficacy and Safety of JZP-110 for the Treatment of Excessive Sleepiness in Patients with Obstructive Sleep Apnea</i>                             | P. Schweitzer et al | <b>Poster presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 PM - 7:00 p.m.</li> <li>• Abstract Number: 0641</li> <li>• Poster Number: 349</li> <li>• Session ID: P36</li> </ul> |
| <i>A Phase 3, Randomized, Placebo-Controlled, Double-Blind, Multicenter, 12-Week Study of the Safety and Efficacy of JZP-110 in the Treatment of Excessive Sleepiness in Patients with Obstructive Sleep Apnea: SF-36 and EQ-5D-5L Measures</i> | H. Benes et al      | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 PM - 7:00 p.m.</li> </ul>   |

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|  |                  | <ul style="list-style-type: none"> <li>• Abstract Number: 0642</li> <li>• Poster Number: 350</li> <li>• Session ID: P36</li> </ul>  |
| <i>Function and Work Productivity Measures in a Phase 3, Randomized, Placebo-Controlled, Double-Blind, Multicenter, 12-Week Study of the Safety and Efficacy of JZP-110 for the Treatment of Excessive Sleepiness in Patients with Obstructive Sleep Apnea</i> | R. Bogan et al   | <b>Poster presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0638</li> <li>• Poster Number: 110</li> <li>• Session ID: P28</li> </ul> |
| <i>A Phase 3, Placebo-Controlled, Randomized-Withdrawal, Double-Blind, 6-Week Multicenter Study of the Safety and Efficacy of JZP-110 for the Treatment of Excessive Sleepiness in Participants with Obstructive Sleep Apnea</i>                               | P. Strollo et al | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0644</li> <li>• Poster Number: 352</li> <li>• Session ID: P36</li> </ul> |

### Sodium Oxybate / Narcolepsy-related Oral and Poster Presentations

| <b>Presentation Title</b>  | <b>Author</b>   | <b>Presentation Date/Time</b>   |
|--|-----------------|---|
| <i>A Double-Blind, Placebo-Controlled, Randomized-Withdrawal, Multicenter Study on the Efficacy and Safety of Sodium Oxybate in Pediatric Subjects with Narcolepsy with Cataplexy</i>  | G. Plazzi et al | <b>Oral Presentation</b> <ul style="list-style-type: none"> <li>• Monday, June 5, 2017; 11:45 a.m. - 12:00 p.m.</li> <li>• Abstract Number: 0645</li> <li>• Session Number: O03</li> </ul> <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Monday, June 5, 2017; 5:00 - 7:00 p.m.</li> <li>• Poster Number: 181</li> <li>• Session Number: P18</li> </ul> |
| <i>Efficacy of Sodium Oxybate for Treatment of Excessive Daytime Sleepiness in Narcolepsy: Meta-Analysis of Randomized Controlled Trials</i>   | M. Erman et al  | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0674</li> <li>• Poster Number: 105</li> <li>• Session Number: P28</li> </ul>   |
| <i>Factors Associated with Use of Different Narcolepsy Medications and Medication Discontinuation Rates: Findings from the Nexus Narcolepsy Registry</i>   | D. Pasta et al  | <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Tuesday Jun 6, 2017 5:00 - 7:00 p.m.</li> <li>• Abstract Number: 0676</li> <li>• Poster Number: 107</li> <li>• Session ID: P28</li> </ul>   |
| <i>Incidence and Duration of Common, Early-Onset, Treatment-Emergent Adverse Events Occurring During Two Randomized, Placebo-Controlled, Phase 3 Studies of Sodium Oxybate for the Treatment of Excessive Sleepiness in Patients with Narcolepsy</i> | A. Husain et al | <b>Oral Presentation</b> <ul style="list-style-type: none"> <li>• Wednesday, June 7, 2017; 2:45 - 3:00 p.m.</li> <li>• Abstract Number: 0650</li> <li>• Session Number: O29</li> </ul> <b>Poster Presentation</b> <ul style="list-style-type: none"> <li>• Tuesday, June 6, 2017 5:00 - 7:00 p.m.</li> <li>• Poster Number: 113</li> <li>• Session Number: P28</li> </ul>     |

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|---|-----------------------|--|
| <p><i>Assessing the Benefits of Sodium Oxybate (SXB) on Functioning, Productivity and Health-Related Quality of Life in People with Narcolepsy: Findings from the Nexus Narcolepsy Registry</i></p> | <p>K. Villa et al</p> | <p><b>Oral Presentation</b></p> <ul style="list-style-type: none"> <li>• Wednesday, June 7, 2017 3:00 - 3:15 p.m.</li> <li>• Abstract Number: 0651</li> <li>• Session Number: O29</li> </ul> <p><b>Poster Presentation</b></p> <ul style="list-style-type: none"> <li>• Tuesday, June 6, 2017 5:00 - 7:00 p.m.</li> <li>• Poster Number: 114</li> <li>• Session Number: P28</li> </ul> |
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Full details of the APSS annual SLEEP meeting can be found at <http://www.sleepmeeting.org/>.

#### **About JZP-110**

JZP-110 is a selective dopamine and norepinephrine reuptake inhibitor (DNRI) in late-stage development for treatment of excessive sleepiness in adult patients with narcolepsy or OSA. In 2014, Jazz Pharmaceuticals acquired a world-wide license (except in certain countries in Asia) to develop and commercialize JZP-110 from SK Biopharmaceuticals, which discovered the compound. Jazz Pharmaceuticals has worldwide development, manufacturing, and commercialization rights to JZP-110, excluding certain jurisdictions in Asia. SK Biopharmaceuticals maintains rights in Korea, Japan, China, Taiwan, Singapore, Indonesia, India, Philippines, Thailand, Malaysia, Vietnam, and Hong Kong. JZP-110 has orphan drug designation in the United States for narcolepsy.

Across the entire JZP-110 development program, over 2,000 subjects have enrolled in 20 studies. The JZP-110 Phase 3 clinical program includes one study evaluating excessive sleepiness in adult patients with narcolepsy (TONES 2), two studies evaluating excessive sleepiness in adult patients with OSA (TONES 3 and TONES 4), and an open-label, long-term safety and maintenance of efficacy study (TONES 5). Enrollment is complete in all studies that are expected to support Jazz Pharmaceuticals' planned JZP-110 New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) in late 2017.

#### **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 in narcolepsy. Xyrem may only be dispensed to patients enrolled in the Xyrem REMS Program. Xyrem was first approved in the U.S. in 2002, based on clinical trial data in adults. The current United States Product Insert for Xyrem indicates that safety and effectiveness in pediatric patients have not been established. Jazz plans to submit a supplemental NDA to FDA in the fourth quarter of 2017 with revised proposed labeling to include data for use in pediatric patients, consistent with the FDA's pediatric written request.

#### **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 central nervous system 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 REMS Program, using the central pharmacy that is specially certified. Prescribers and patients must enroll in the program. For further information go to [www.XYREMREMS.com](http://www.XYREMREMS.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.

Caution should be used 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. The rapid onset of sedation, coupled with the amnesic features of Xyrem, particularly when combined with alcohol, has proven to be dangerous for the voluntary and involuntary user (e.g. assault victim). Patients should be monitored for emergent or increased depression and suicidality and for impaired motor/cognitive function. Episodes of sleepwalking should be fully evaluated and appropriate interventions considered. The amount of daily sodium intake in each dose of Xyrem should be considered in patients sensitive to salt intake. The most common adverse reactions were nausea, dizziness, vomiting, somnolence, enuresis, and tremor.

Please [click here](#) to see the full Prescribing Information for Xyrem, including BOXED Warning.

#### **About the Nexus Narcolepsy Registry**

The Nexus Narcolepsy Registry is an effort by Wake Up Narcolepsy and the narcolepsy research and advocacy communities, in collaboration with Jazz Pharmaceuticals, to collect de-identified data from a large group of people with narcolepsy over several years in order to shed light on the impact of the condition on people's lives. To learn more about the registry, please visit [www.narcolepsyregistry.com](http://www.narcolepsyregistry.com).

#### **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 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](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 company's commitment to advancing meaningful treatments that address unmet needs in sleep medicine, the company's plans for submission of an NDA for JZP-110 to the FDA, the company's plans for submission of a supplemental NDA to the FDA for the use of Xyrem in pediatric patients, 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; the regulatory approval process, including the risks that the company may be unable to obtain approval by the FDA for JZP-110 or for its planned supplemental NDA for Xyrem in a timely manner or at all; effectively commercializing the company's products and product candidates; 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 March 31, 2017 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.



To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/jazz-pharmaceuticals-to-present-phase-3-data-on-jzp-110-an-investigational-treatment-for-excessive-sleepiness-in-patients-with-narcolepsy-and-with-obstructive-sleep-apnea-during-31st-annual-sleep-meeting-300463450.html>

SOURCE Jazz Pharmaceuticals plc

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