Jazz Pharmaceuticals Provides Update on Cannabidiol Oral Solution Phase 3 Trial in Japan in Treatment-Resistant Epilepsies

August 22, 2024

DUBLIN, Aug. 22, 2024 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced top-line results from the Phase 3 open-label, single-arm trial in Japan evaluating the safety and efficacy of cannabidiol oral solution (marketed as Epidiolex®/Epidyolex® globally) as an adjunctive treatment for seizures associated with Lennox-Gastaut syndrome (LGS), Dravet syndrome (DS) or tuberous sclerosis complex (TSC). The trial did not meet the primary efficacy endpoint of a pre-specified percentage change in indication-associated seizure frequency during the treatment period (up to 16 weeks) compared to baseline in Japanese pediatric patients; however, numeric improvements were observed in the primary and several secondary endpoints. No new safety signals were observed in the trial.

"We are confident in the overall clinical profile of *Epidyolex*, which has been established in five Phase 3 clinical trials in more than 900 patients. We believe the totality of the *Epidyolex* global data, including the findings from this trial, supports advancement of the program in Japan," said Rob Iannone, M.D., M.S.C.E., executive vice president, global head of research and development of Jazz Pharmaceuticals. "We are continuing to collect data in Japanese patients and plan to engage with regulatory authorities in Japan regarding a potential new drug application (JNDA). We recognize the significant unmet need for patients in Japan living with rare epilepsies and thank the investigators, patients and caregivers who are involved in this trial."

About the Phase 3 Trial

The Phase 3 open-label, single-arm clinical trial investigates the safety and efficacy of cannabidiol oral solution (GWP42003-P) for the treatment of seizures associated with LGS, DS or TSC in Japanese pediatric patients. The trial includes a pre-specified primary efficacy outcome measuring the percentage change in indication-associated seizure frequency during the treatment period (up to 16 weeks) compared to baseline in 62 patients ≥ 1 to ≤ 18 years of age. The trial design includes a treatment period consisting of an initial 2-week titration period followed by a 14-week maintenance period; and a period evaluating safety for up to 52 weeks of treatment. The trial remains ongoing to collect efficacy and safety data in pediatric and adult Japanese patients.

About Cannabidiol

Cannabidiol, 100 mg/mL oral solution, a prescription, plant-derived cannabis-based medicine approved by the U.S. Food and Drug Administration (FDA) for use in the U.S., and the European Medicines Agency (EMA) for use in the European Union, is an oral solution which contains highly purified cannabidiol (CBD). In the U.S., cannabidiol, under the tradename *Epidiolex*, is indicated for the treatment of seizures associated with Lennox-Gastaut syndrome (LGS), Dravet syndrome or tuberous sclerosis complex (TSC) in patients one year of age and older. Cannabidiol has also received approval in the European Union, under the tradename *Epidyolex*, for adjunctive use in conjunction with clobazam to treat seizures associated with LGS and Dravet syndrome in patients two years and older, and for adjunctive use to treat seizures associated with TSC, in patients two years of age and older.

About Jazz Pharmaceuticals

Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing life-changing medicines for people with serious diseases — often with limited or no therapeutic options. We have a diverse portfolio of marketed medicines, including leading therapies for sleep disorders and epilepsy, and a growing portfolio of cancer treatments. Our patient-focused and science-driven approach powers pioneering research and development advancements across our robust pipeline of innovative therapeutics in oncology and neuroscience. Jazz is headquartered in Dublin, Ireland with research and development laboratories, manufacturing facilities and employees in multiple countries committed to serving patients worldwide. Please visit www.jazzpharmaceuticals.com for more information.

Caution Concerning Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to potential timing of data availability and regulatory engagement related to the Phase 3 clinical trial in Japan 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 pharmaceutical product development, 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' Annual Report on Form 10-K for the year ended December 31, 2023, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended March 31, 2024, 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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