Jazz Pharmaceuticals to Present New Data Demonstrating Improved Epilepsy Outcomes with Epidiolex®/Epidyolex® (cannabidiol) at the European Epilepsy Congress 2024

September 05, 2024

Data from eight abstracts to be presented, including results of the BECOME (BEhavior, COgnition and More with Epidyolex®) Caregiver Survey on the real-world outcomes of Epidyolex® (cannabidiol) treatment on patients with Lennox-Gastaut Syndrome (LGS) and Dravet Syndrome (DS)

DUBLIN, Sept. 5, 2024 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the Company will present eight abstracts at the 15th European Epilepsy Congress (EEC), including a subgroup analysis of the BECOME (*BEhavior, COgnition and More with Epidyolex®*) Caregiver Survey, a survey of caregivers of patients with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS) reporting outcomes in patients receiving Epidiolex/Epidyolex (cannabidiol, 100mg/mL oral solution) and concomitant clobazam. Results showed most caregivers reported patient improvements in seizure and non-seizure outcomes since initiating *Epidyolex*. In addition, post-hoc analyses of pooled data from 215 participants from the GWPCARE3 and GWPCARE4 *Epidyolex* Phase 3 trials will be presented, demonstrating thresholds for clinically meaningful reductions in drop seizures in patients with LGS. EEC is being held September 7-11, 2024, in Rome, Italy.

"Presentations at EEC this year continue to build the scientific evidence in support of *Epidyolex*, with data from more than 2,000 patient experiences, demonstrating improved understanding and real-world outcomes reflecting the treatment's holistic impact -- not only for patients but importantly their families and caregivers as well," said Tomas Skacel, vice president of medical affairs, Europe and international, at Jazz Pharmaceuticals. "In addition to seizure outcomes, the BECOME subgroup analysis underscores the impact of non-seizure benefits of *Epidyolex* and the potential to improve the day-to-day lives for both patients and their loved ones."

Presentation highlights include:

- **Epidyolex Beyond-Seizure Benefits (P251):** Results from the BECOME subgroup analysis indicating that treatment with *Epidyolex* (for ≥3 months) and concomitant clobazam, in both pediatric (2–17 years) and adult (≥18 years) patients with LGS or DS, led to caregiver-reported improvements in both seizure frequency (87%), seizure severity (81%) and weekly seizure-free days (68%). Notably, results reported improvements in at least one item for non-seizure-related domains, including alertness, cognition and executive function (84%) and language and communication in non-verbal (81%) and verbal (76%) patients. In addition, 94% of the 243 caregivers surveyed indicated they planned to continue with *Epidyolex* treatment.
- **Epidyolex Efficacy in Drop Seizures (P424):** Results from a post-hoc analysis of data from pivotal trials GWPCARE3 and GWPCARE4, in patients with LGS who received *Epidyolex* for 14 weeks, evaluated the threshold for a clinically meaningful reduction in drop seizures associated with Caregiver Global Impression of Change (CGIC). The analysis showed that of 215 patients analyzed, 67 (31%) reported a 'much improved or better' CGIC score and 129 (60%) reported a 'slightly improved or better' CGIC score; and that the best threshold for a clinically important response (CIR) were reductions in drop seizures of −30.6% and −49.6% for 'slightly improved or better' and 'much improved or better', respectively. For those receiving concomitant clobazam, CIR thresholds were relatively consistent between subgroups and the overall population.
- **Need for Improved Diagnosis (P293 & P292):** Results from two analyses of real-world data from the Adelphi LGS and DS Disease-Specific Programmes™, which are independent real-world evidence databases. The analyses describe the clinical characteristics of 276 patients with LGS and 212 patients with DS from across Europe (France, Germany, Italy, Spain and the UK). These analyses, which were not limited to *Epidyolex*, suggest:
    - Initial misdiagnosis is common in both patients with LGS and DS (53.7% of pediatric and 81.3% of adult patients were diagnosed with another seizure type prior to LGS; and 66.7% of pediatric and 79.3% of adult patients were diagnosed with another seizure type prior to DS) – highlighting the need for improved diagnosis.
    - Following initial monotherapy, the majority of patients go on to receive at least two further anti-seizure medications (83.2% of pediatric and 82.7% of adult LGS patients subsequently received ≥2 medications; and 83.9% of pediatric and 71.4% of adult DS patients subsequently received ≥2 medications) – highlighting the need to identify effective early line treatment options.
- **Effect of a Ketogenic Diet:** A late-breaking abstract featuring the results of a post-hoc analysis of data from three randomized, controlled trials: two in LGS patients (GWPCARE3/NCT02224560 and GWPCARE4/NCT02224690), and one in DS patients (GWPCARE2/NCT02224703), who received *Epidyolex* for 14 weeks, which explored the effect of a ketogenic diet (KD)*, on cannabidiol plasma concentration and seizure reduction in patients with LGS or DS treated with *Epidyolex* in conjunction with clobazam. The analysis found no substantial differences in cannabidiol plasma concentration or seizure responses between patients in KD and non-KD groups. Further studies are needed to confirm these findings.

** KD; A form of non-pharmacological treatment used in attempts to provide seizure control for patients who experience pharmacoresistance.*

The EEC 2024 abstracts are available at: www.ilae.org/congresses/15th-european-epilepsy-congress/section/sectionid/2.

All Jazz Pharmaceuticals data will be featured at the in-person poster presentations, occurring Sunday, 8 September through Tuesday, 10 September, with investigators available 13:30-15:00 daily. A full list of the presentations are:

Presentation Title	Lead Author	Poster Number
Clinically meaningful reduction in drop seizures in patients with Lennox-Gastaut syndrome treated with cannabidiol	N. Specchio	P424
Treatment Outcomes With Cannabidiol in Patients ≥2 Years of Age With Lennox-Gastaut Syndrome or Dravet Syndrome Receiving Concomitant Clobazam: A Subgroup Analysis of BECOME, a Caregiver Survey	K. Vyas	P251
Patient Characteristics and Treatment Patterns in Patients With Dravet Syndrome: Real-world Evidence from a Cross-sectional Survey of Physicians in Europe	R. Chin	P293
Patient Characteristics and Treatment Patterns in Patients With Lennox-Gastaut Syndrome: Real-world Evidence from a Cross-sectional Survey of Physicians in Europe	J. Lin	P292
Real-world Insights on the Use of Cannabidiol in Adults With Lennox-Gastaut syndrome, Dravet Syndrome, or Tuberous Sclerosis Complex: A Physician Survey	K. Vyas	P290
Efficacy and Safety of Cannabidiol for Seizures Associated With Tuberous Sclerosis Complex in Paediatric and Adult Patients From GWPCARE6: A Phase 3 Trial With Open-Label Extension	T. Saurer	P275
Change in Antiseizure and Anxiolytic Medications Pre- and Post-Cannabidiol Initiation	T. Greco	P291
Late-breaking abstract		
The effect of a ketogenic diet on cannabidiol plasma concentration and seizure reduction in patients with Lennox-Gastaut syndrome or Dravet syndrome: post-hoc analysis of data from the randomised controlled trials	C Johannessen Landmark	P323

About Epidiolex®/Epidyolex® (cannabidiol)
Epidiolex®/Epidyolex® (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 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 is a global biopharma company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing potentially 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.

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