Jazz Pharmaceuticals Announces Initiation of Phase 3 Trial Evaluating Epidiolex®/Epidyolex® (cannabidiol) for Patients with Epilepsy with Myoclonic-Atonic Seizures

August 18, 2022

Trial is the first randomized, controlled clinical trial evaluating cannabidiol to treat children and adolescents living with a developmental and epileptic encephalopathy known as Epilepsy with Myoclonic-Atonic Seizures

DUBLIN, Aug. 18, 2022 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced it has initiated a new Phase 3 trial to investigate the efficacy and safety of Epidiolex® (cannabidiol), known as Epidyolex® in Europe, in children and adolescents with Epilepsy with Myoclonic-Atonic Seizures (EMAS). The randomized, double-blind, placebo-controlled study will investigate EMAS-associated seizure frequency over the 14-week treatment period compared to baseline. The Company's cannabidiol is not currently approved in the United States or European Union for the treatment of EMAS.

EMAS – also known as Myoclonic Astatic Epilepsy (MAE) or Doose Syndrome – is a developmental and epileptic encephalopathy that begins in early childhood. EMAS accounts for between one and two percent of all childhood-onset epilepsies.[1] Seizures in children with EMAS are often difficult to treat and may not respond well to medication.[2]

"Given there are numerous treatment-resistant epilepsy syndromes, epileptologists often look for efficacy by seizure type, most of which have no syndrome-specific approved treatment. An EMAS indication would provide support for the use of Epidiolex in a fourth indication of a distinct, generalized seizure type, myoclonic-atonic seizures," said Rob Iannone, M.D., M.S.C.E., executive vice president, global head of research and development of Jazz Pharmaceuticals. "Jazz is committed to continuing to generate clinical study data and real-world evidence to further support the utility of the Company's cannabidiol across a broad range of difficult-to-treat seizure types."

The Phase 3 pivotal trial GWEP20238 (NCT05288283) will be run in two parts and will enroll children and adolescent participants (ages 1-18) at 30 global sites. Part A will assess efficacy and safety of cannabidiol compared to placebo as an adjunctive treatment for children and adolescents with myoclonic-atonic seizures. Upon completion of Part A, participants will have an option to continue in a 54-week open-label extension (Part B).

The Phase 3 trial was initiated based on preliminary data from our clinical development program, including real-world evidence, that supports cannabidiol as an effective therapy for the treatment of myoclonic-atonic-associated seizures.

NOTES TO EDITORS:
About EPIDIOLEX®/EPIDYOLEX® (cannabidiol)

EPIDIOLEX®/EPIDYOLEX® (cannabidiol), is a prescription, plant-derived cannabis-based medicine administered as an oral solution which contains highly purified cannabidiol (CBD). Cannabidiol, the active ingredient in EPIDIOLEX, is a cannabinoid that naturally occurs in the *Cannabis sativa* L. plant. The precise mechanisms by which EPIDIOLEX exerts its anticonvulsant effect in humans are unknown.

For the EU: Please refer to the EPIDYOLEX Summary of Product Characteristics and Full Prescribing Information for details on the therapeutic indications and safety information about this product – which can be found here.

For the UK: Please refer to the EPIDYOLEX Summary of Product Characteristics and Full Prescribing Information for details on the therapeutic indications and safety information about this product – which can be found here.

For the US: Please refer to the EPIDIOLEX Full Prescribing Information for details on therapeutic indications and safety information about this product—which can be foundhere.

About Jazz Pharmaceuticals plc

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 and novel product candidates, from early- to late-stage development, in neuroscience and oncology. Within these therapeutic areas, we are identifying new options for patients by actively exploring small molecules and biologics, and through innovative delivery technologies and cannabinoid science. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries. For more information, please visit www.jazzpharmaceuticals.com and follow @JazzPharma on Twitter.

Caution Concerning Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the potential for *Epidiolex* as a treatment for people with EMAS and the potential impact on that community 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; 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 Jazz Pharmaceuticals' Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 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.

Media Contact:

Kristin Bhavnani
Head of Global Corporate Communications
Jazz Pharmaceuticals plc
CorporateAffairsMediaInfo@jazzpharma.com
Ireland +353 1 637 2141
U.S. +1 215 867 4948

Investors:

Andrea N. Flynn, Ph.D.
Vice President, Head, Investor Relations
Jazz Pharmaceuticals plc
investorinfo@jazzpharma.com
Ireland, +353 1 634 3211
U.S. +1 650 496 2717

References

[1] Hinokuma N, Nakashima M, Asai H, et al. Clinical and genetic characteristics of patients with Doose syndrome. *Epilepsia Open*. 2020; 5: 442-450

[2] Kelley SA, Kossoff E. Doose syndrome (myoclonic-astatic epilepsy): 40 years of progress. *Developmental Medicine & Child Neurology.* 2010; 52:11, 988-993

View original content to download multimedia:https://www.prnewswire.com/news-releases/jazz-pharmaceuticals-announces-initiation-of-phase-3-trial-evaluating-epidiolexepidyolex-cannabidiol-for-patients-with-epilepsy-with-myoclonic-atonic-seizures-301608430.html

SOURCE Jazz Pharmaceuticals plc