



GW Pharmaceuticals receives approval for EPIDYOLEX® (cannabidiol) from the MHRA for the treatment of seizures associated with tuberous sclerosis complex

August 10, 2021

It is estimated that between 3,700 and 11,000 people in the UK live with tuberous sclerosis complex (TSC)(1) TSC causes epilepsy in up to 85% of patients, and up to 60% of those patients do not respond to standard anti-seizure medicines(2),(3),(4)

Approval in TSC represents a new indication for GW's cannabidiol in the UK

LONDON, Aug. 10, 2021 /PRNewswire/ -- GW Pharmaceuticals ("GW"), a world leader in discovering, developing and delivering regulatory approved cannabis-based medicines and now part of Jazz Pharmaceuticals plc (Nasdaq: JAZZ), today announces that the UK Medicines and Healthcare Products Regulatory Agency (MHRA) has approved a new indication for GW's cannabidiol as an adjunctive treatment of seizures associated with tuberous sclerosis complex (TSC), for patients two years of age and older.



GW was initially granted marketing authorisation for this medicine in the UK in September 2019 as an adjunctive therapy for seizures associated with Lennox Gastaut syndrome (LGS) or Dravet syndrome, in conjunction with clobazam, for patients two years of age and older. GW also received positive recommendation from the UK's National Institute for Health and Care Excellence (NICE) for this medicine in November 2019 to receive routine reimbursement from NHS England. This authorisation for TSC, represents the fourth approved indication of a cannabis-based medicine in the UK for GW and underlines GW's commitment to the UK and regulatory approved cannabis-based medicines.

"We are delighted by today's decision from the MHRA for this new indication, which represents a significant step forward for TSC patients in the UK, many of whom may benefit from this new treatment," said Chris Tovey, Executive Vice President, Chief Operating Officer and Managing Director, Europe & International at Jazz Pharmaceuticals. "The UK is a significant country for us for many reasons, and we are proud to be able to offer a medicine, that has been developed and is manufactured here, to even more patients across the UK. This authorisation, expanding the label, is further proof of our continued commitment to the UK and the pioneering research and development into regulatory approved cannabis-based medicines we have conducted here."

Commenting on the MHRA approval of GW's cannabidiol, Tuberous Sclerosis Association (TSA) Chief Executive Louise Fish said: "One in every two people living with TSC-related epileptic seizures has difficulty to treat epilepsy that does not respond to traditional anti-epileptic drugs. We are excited to see this new medicine approved by the MHRA, which gives people living with TSC and their families hope. We desperately need further options for clinicians who are managing seizures associated with TSC, and we therefore welcome this decision by the MHRA."

The approval, made through the European Commission Decision Reliance Procedure (ECDRP), is based on data from a positive Phase 3 safety and efficacy study evaluating 25 mg/kg/day of GW's cannabidiol. The study met its primary endpoint, which was the reduction in seizure frequency compared to baseline of cannabidiol vs placebo, with seizure reduction of 49% in patients taking cannabidiol 25 mg/kg/day compared with 27% for placebo ($p=0.0009$). All key secondary endpoints were supportive of the effects on the primary endpoint. The safety profile observed was consistent with findings from previous studies, with no new safety risks identified. GW's development programme represents the only well-controlled clinical evaluation of a cannabinoid medication for patients with refractory epilepsy.

TSC is a condition that causes mostly benign tumours to grow in vital organs of the body, including the brain, skin, heart, eyes, kidneys and lungs, and in which epilepsy is the most common neurological feature. TSC is typically diagnosed in childhood.⁽⁵⁾ It is estimated that between 3,700 and 11,000 people in the UK live with TSC.⁽¹⁾

The approval from the MHRA applies in England, Wales and Scotland and follows the recent approval of the product in all 27 countries of the European Union, alongside Norway, Iceland and Liechtenstein (including Northern Ireland). Following this approval, GW will work with the relevant stakeholders in the UK, including NICE, to secure reimbursement for eligible patients.

ADDITIONAL INFORMATION

About GW Pharmaceuticals Limited

GW Pharmaceuticals (GW), a subsidiary of Jazz Pharmaceuticals plc, has established a world-leading position in cannabinoid science and medicine. Founded over two decades ago in response to significant unmet patient need, patients remain our key focus and improving their quality of life, our motivation. GW's pioneering work has led to the regulatory approval of world first, potentially life changing, cannabis-based medicines. Our continued dedication has resulted in the treatment of thousands of patients with our medicines in the UK and around the world. In 2021, GW was received the Queens Award for Enterprise in Innovation in recognition of its innovative and ground-breaking work in developing prescription cannabis-based medicines. GW was acquired by Jazz Pharmaceuticals in May 2021.

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.

About EPIDIOLEX®/EPIDYOLEX® (cannabidiol)

EPIDIOLEX®/EPIDYOLEX® (cannabidiol), the first 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 Commission (EC) for use in the European Union, is an oral solution which contains highly purified cannabidiol (CBD). In the U.S., 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. EPIDIOLEX® has 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. EPIDYOLEX® has received Orphan Drug Designation from the European Medicines Agency (EMA) for the treatment of seizures associated LGS, Dravet syndrome, and Tuberous Sclerosis Complex (TSC).

About Tuberous Sclerosis Complex (TSC)

Tuberous Sclerosis Complex (TSC) is a rare genetic condition that has an estimated prevalence in the EU of 10 in 100,000.⁽⁶⁾ The condition causes mostly benign tumours to grow in vital organs of the body including the brain, skin, heart, eyes, kidneys and lungs and is a leading cause of genetic epilepsy.^{(5),(6)} The onset of epilepsy in TSC often occurs in the first year of life with patients initially suffering from predominantly focal seizures or infantile spasms. It is associated with an increased risk of autism and intellectual disability.⁽¹⁾ The severity of the condition can vary widely. In some children the disease is very mild, while others may experience life-threatening complications.^{(7),(8)}

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5. NIH Tuberous Sclerosis Fact Sheet. <https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Tuberous-Sclerosis-Fact-Sheet>.
6. Prevalence and incidence of rare diseases: Bibliographic data. https://www.orpha.net/orphacom/cahiers/docs/GB/Prevalence_of_rare_diseases_by_alphabetical_list.pdf
7. TS Alliance Website. <https://www.tsalliance.org/>. Accessed August 2021
8. de Vries PJ, Belousova E, Benedik MP, et al. TSC-associated neuropsychiatric disorders (TAND): findings from the TOSCA natural history study. *Orphanet J Rare Dis*. 2018;13(1):157.

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