FDA Approves EPIDIOLEX® (cannabidiol) Oral Solution to Treat Seizures Associated with Tuberous Sclerosis Complex

August 3, 2020

– Age-range for all EPIDIOLEX indications expanded to include patients one year of age and older –

– Third indication approved for EPIDIOLEX, the only FDA-approved Cannabidiol (CBD) medicine –

CARLSBAD, Calif., Aug. 03, 2020 (GLOBE NEWSWIRE) -- GW Pharmaceuticals plc (Nasdaq: GWPH), the world leader in the science, development, and commercialization of cannabinoid prescription medicines, along with its U.S. subsidiary Greenwich Biosciences, Inc., today announced the U.S. Food and Drug Administration (FDA) has approved EPIDIOLEX® (cannabidiol) oral solution to treat seizures associated with tuberous sclerosis complex (TSC) in patients one year of age and older. Along with this new indication, the age range has been expanded to include patients one year of age and older who experience seizures associated with Lennox-Gastaut syndrome (LGS) or Dravet syndrome. TSC is a rare disease that causes benign tumors to grow in vital organs of the body and is a leading cause of genetic epilepsy.[1] EPIDIOLEX, the first plant-derived cannabinoid prescription medicine and the only FDA-approved form of cannabidiol (CBD), was initially approved by the FDA in June 2018 for the treatment of seizures associated with LGS or Dravet syndrome in patients two years of age and older. GW has also received approval for this medicine in the European Union under the tradename EPIDYOLEX® and a TSC EMA submission is currently under review.

“FDA approval of this new indication is exciting news for those with refractory seizures due to tuberous sclerosis complex,” said Justin Gover, GW’s Chief Executive Officer. “Since EPIDIOLEX is already available to patients by physician’s prescription, patients with TSC can immediately access the medication. This label expansion, including the expansion of the age range in all approved indications, further demonstrates that the FDA process can continue to enable broader patient access to appropriately tested regulatory approved cannabinoid medicines. It also provides hope for these patients and their families and is yet another important milestone for EPIDIOLEX as a first-in-class antiepileptic drug.”

“Based on previous positive trial results in TSC patients, EPIDIOLEX may become an important treatment option for patients. It is a new tool in the toolbox for physicians and could meet a significant unmet need," said Elizabeth Thiele, M.D., Ph.D., Director of the Herscot Center for Tuberous Sclerosis Complex at Massachusetts General Hospital, Professor of Neurology at Harvard Medical School and clinical investigator. “Nearly two-thirds of individuals with TSC develop treatment-resistant epilepsy and there is a need for new options that may benefit these patients who often try and fail existing treatments.”

The Company anticipates most payers will quickly update their EPIDIOLEX coverage policies to include TSC at access levels similar to that of LGS and Dravet syndrome.

“FDA approval of EPIDIOLEX in TSC is a tremendous step forward and our community applauds this positive development,” said Kari Luther Rosbeck, President and CEO of the Tuberous Sclerosis Alliance. “One of the most challenging and frustrating aspects of TSC are seizures that cannot be effectively controlled by existing medications. New treatment options are desperately needed, and this approval adds another option for those impacted by this difficult disease.”

FDA approval includes a recommended maintenance dose of 25 mg/kg/day for TSC patients, which is supported by data from a Phase 3 safety and efficacy study evaluating 25 mg/kg/day of EPIDIOLEX. The study met its primary endpoint, which was the reduction in seizure frequency compared to baseline of Epidiolex vs placebo, with seizure reduction of 48 percent in patients taking Epidiolex 25 mg/kg/day compared with 24 percent for placebo ($p<0.01$). All key secondary endpoints were supportive of the effects on the primary endpoint. The most common adverse events in those receiving EPIDIOLEX in the study (≥10 percent and greater than placebo) included diarrhea; transaminase elevations; decreased appetite; somnolence; pyrexia; and vomiting. The safety profile observed in this study was generally comparable to that observed in prior studies of Epidiolex.

About Tuberous Sclerosis Complex (TSC)

Tuberous sclerosis complex (TSC) is a rare genetic condition that affects approximately 50,000 individuals in the U.S. and nearly one million people worldwide.[1] At least two children born each day will develop TSC, with an estimated prevalence of one in 6,000 newborns.[5] The condition causes mostly benign tumors to grow in vital organs of the body including the brain, skin, heart, eyes, kidneys and lungs [2] and is a leading cause of genetic epilepsy.[3] People with TSC may experience a variety of seizure types. One of the most common is infantile spasms that typically present in the first year of life; focal (or partial) seizures are also very common.[4] TSC is associated with an increased risk of autism and intellectual disability[5] and the severity of the condition can vary widely. In some children the disease is very mild, while others may experience life-threatening complications.[6] Epilepsy is present in about 85 percent of patients with TSC and may progress to become intractable to medication.[1,2,3] More than 60 percent of individuals with TSC do not achieve seizure control[4] with standard treatments such as antiepileptic drugs, epilepsy surgery, ketogenic diet, or vagus nerve stimulation[2] compared to 30-40 percent of individuals with epilepsy who do not have TSC who are drug resistant.[6,7]

About GW Pharmaceuticals plc and Greenwich Biosciences, Inc.

Founded in 1998, GW is a biopharmaceutical company focused on discovering, developing and commercializing novel therapeutics from its proprietary cannabinoid product platform in a broad range of disease areas. The Company’s lead product, EPIDIOLEX ® (cannabidiol) oral solution is commercialized in the U.S. by its U.S. subsidiary Greenwich Biosciences 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. This product has received approval in the European Union under the tradename EPIDYOLEX® for the adjunctive treatment of seizures associated with Lennox-Gastaut syndrome (LGS) or

Dravet syndrome in conjunction with clobazam in patients two years and older and is under EMA review for the treatment of TSC. The Company is also carrying out a Phase 3 trial in Rett syndrome and has a deep pipeline of additional cannabinoid product candidates, including nabiximols, for which the Company is advancing multiple late-stage clinical programs in order to seek FDA approval in the treatment of spasticity associated with multiple sclerosis and spinal cord injury, as well as for the treatment of PTSD. The Company has additional cannabinoid product candidates in Phase 2 trials for autism and schizophrenia. For further information, please visit www.gwpharm.com.

About EPIDIOLEX® (cannabidiol) oral solution

EPIDIOLEX® (cannabidiol) oral solution, a pharmaceutical formulation of highly purified cannabidiol (CBD), is the first in a new class of anti-epileptic medications with a novel mechanism of action, and the first prescription, plant-derived cannabis-based medicine approved by the U.S. Food and Drug Administration (FDA). 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. EPIDIOLEX/EPIDYOLEX has received Orphan Drug Designation from the FDA and the EMA for the treatment of seizures associated with Dravet syndrome, LGS and TSC, each of which are severe childhood-onset, drug-resistant syndromes and is under EMA review for the treatment of TSC. Important safety and prescribing information for EPIDIOLEX is available at Epidiolex.com.

Important Safety Information & Indications

CONTRAINDICATION: HYPERSENSITIVITY

EPIDIOLEX (cannabidiol) oral solution is contraindicated in patients with a history of hypersensitivity to cannabidiol or any ingredients in the product.

WARNINGS & PRECAUTIONS

Hepatocellular Injury:

EPIDIOLEX can cause dose-related transaminase elevations. Concomitant use of valproate and elevated transaminase levels at baseline increase this risk. Transaminase and bilirubin levels should be obtained prior to starting treatment, at one, three, and six months after initiation of treatment, and periodically thereafter, or as clinically indicated. Resolution of transaminase elevations occurred with discontinuation of EPIDIOLEX, reduction of EPIDIOLEX and/or concomitant valproate, or without dose reduction. For patients with elevated transaminase levels, consider dose reduction or discontinuation of EPIDIOLEX or concomitant medications known to affect the liver (e.g., valproate or clobazam). Dose adjustment and slower dose titration is recommended in patients with moderate or severe hepatic impairment. Consider not initiating EPIDIOLEX in patients with evidence of significant liver injury.

Somnolence and Sedation:

EPIDIOLEX can cause somnolence and sedation that generally occurs early in treatment and may diminish over time; these effects occur more commonly in patients using clobazam and may be potentiated by other CNS depressants.

Suicidal Behavior and Ideation:

Antiepileptic drugs (AEDs), including EPIDIOLEX, increase the risk of suicidal thoughts or behavior. Inform patients, caregivers, and families of the risk and advise to monitor and report any signs of depression, suicidal thoughts or behavior, or unusual changes in mood or behavior. If these symptoms occur, consider if they are related to the AED or the underlying illness.

Withdrawal of Antiepileptic Drugs:

As with most AEDs, EPIDIOLEX should generally be withdrawn gradually because of the risk of increased seizure frequency and status epilepticus.

ADVERSE REACTIONS:

The most common adverse reactions in patients receiving EPIDIOLEX (≥10% and greater than placebo) include transaminase elevations; somnolence; decreased appetite; diarrhea; pyrexia; vomiting; fatigue, malaise, and asthenia; rash; insomnia, sleep disorder and poor-quality sleep; and infections. Hematologic abnormalities were also observed.

PREGNANCY:

EPIDIOLEX should be used during pregnancy only if the potential benefit justifies the potential risk to the fetus. Encourage women who are taking EPIDIOLEX during pregnancy to enroll in the North American Antiepileptic Drug (NAAED) Pregnancy Registry.

DRUG INTERACTIONS:

Strong inducers of CYP3A4 and CYP2C19 may affect EPIDIOLEX exposure. EPIDIOLEX may affect exposure to CYP2C19 substrates (e.g., clobazam, diazepam) or others. Concomitant use of EPIDIOLEX and valproate increases the incidence of liver enzyme elevations. No drug interaction studies have been completed, but case reports suggest a potential for elevations of mammalian target of rapamycin (mTOR) or calcineurin inhibitors when used with EPIDIOLEX. Dosage adjustment of EPIDIOLEX or other concomitant medications may be necessary.

INDICATIONS:

EPIDIOLEX (cannabidiol) oral solution is indicated for the treatment of seizures associated with Lennox-Gastaut syndrome (LGS), Dravet syndrome (DS), or tuberous sclerosis complex (TSC) in patients 1 year of age and older.

Please refer to the EPIDIOLEX full Prescribing Information for additional important information.

Forward-looking statement

This news release contains forward-looking statements that reflect GW's current expectations regarding future events, including statements regarding our goal of bringing EPIDIOLEX® to patients with seizures associated with Tuberous Sclerosis Complex (TSC), the potential for EPIDIOLEX to serve as a new treatment option for patients one year of age and older with seizures associated with TSC, the potential for EPIDIOLEX to serve as a treatment option for patients one year of age and older with seizures associated with Lennox-Gastaut syndrome or Dravet syndrome, the clinical benefits of EPIDIOLEX, and the timing and outcomes of regulatory decisions. Actual events could differ materially from those projected herein and depend on a number of factors, including (inter alia), the risks and uncertainties which can be found in GW's filings with the U.S. Securities and Exchange Commission. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. GW undertakes no obligation to update or revise the information contained in this press release, whether as a result of new information, future events or circumstances or otherwise.

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