FDA NEWS RELEASE

FDA Approves New Indication for Drug Containing an Active Ingredient Derived from Cannabis to Treat Seizures in Rare Genetic Disease

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Today, the U.S. Food and Drug Administration approved Epidiolex (cannabidiol) [CBD] oral solution for the treatment of seizures associated with tuberous sclerosis complex (TSC) in patients one year of age and older. Epidiolex was previously approved for the treatment of seizures associated with two rare and severe forms of epilepsy, Lennox-Gastaut syndrome (LGS) and Dravet syndrome (DS). This is the only FDA-approved drug that contains a purified drug substance derived from cannabis. It is also the second FDA approval of a drug for the treatment of seizures associated with TSC.

CBD is a chemical component of the Cannabis sativa plant. However, CBD does not cause intoxication or euphoria (the "high") that comes from tetrahydrocannabinol (THC). It is THC (and not CBD) that is the primary psychoactive component of cannabis.

"The FDA continues to believe the drug approval process represents the best way to make new medicines, including any drugs derived from cannabis, available to patients in need of appropriate medical therapy such as the treatment of seizures associated with these rare conditions. This paradigm ensures new therapies are safe, effective, and manufactured to a high quality that provides uniform and reliable dosing for patients," said Douglas Throckmorton, M.D., deputy center director for regulatory programs in the FDA's Center for Drug Evaluation and Research. "The agency is committed to supporting rigorous scientific research on the potential medical uses of cannabis-derived products and working with product developers who are interested in bringing patients safe and effective, high quality products."

TSC is a rare genetic disease that causes non-cancerous (benign) tumors to grow in the brain and other parts of the body like the eyes, heart, kidneys, lungs, and skin. TSC usually affects the central nervous system and can result in a combination of symptoms including seizures, developmental delay, and behavioral problems, although the signs and symptoms of the condition, as well as the severity of symptoms, vary widely. TSC affects about 1 in 6,000 people.

Epidiolex's effectiveness for the treatment of seizures associated with TSC was established in a randomized, double-blind, placebo-controlled trial where 148 patients out of a total of 224 in the study received Epidiolex. The study measured the change from baseline in seizure frequency. In the study, patients treated with Epidiolex had a significantly greater reduction in the frequency of seizures during the treatment period than patients who received placebo (inactive treatment). This effect was seen within eight weeks and remained consistent throughout the 16-week treatment period.

The most common side effects that occurred in Epidiolex-treated patients with TSC in the clinical trial were: diarrhea, elevated liver enzymes, decreased appetite, sleepiness, fever, and vomiting. Additional side effects for patients with LGS, DS, or TSC include: liver injury, decreased weight, anemia, and increased creatinine.

Epidiolex must be dispensed with a patient Medication Guide that describes important information about the drug's uses and risks. As is true for all drugs that currently treat epilepsy, including Epidiolex, the most serious risks may include an increase in suicidal thoughts and behavior, or thoughts of self-harm. Patients, their caregivers, and their families should be advised to monitor for any unusual changes in mood or behavior, such as worsening depression, suicidal thoughts or behavior. Patients, caregivers, and families should report behaviors of concern immediately to healthcare providers. Epidiolex also caused liver injury in some patients. Most cases were generally mild, but a risk of rare, but more severe liver injury exists. More severe liver injury can cause nausea, vomiting, abdominal pain, fatigue, anorexia, jaundice, and/or dark urine.

The FDA granted Priority Review designation for this application. The approval of Epidiolex was granted to Greenwich Biosciences Inc., of Carlsbad, California.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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