FDA NEWS RELEASE

FDA Approves First Drug Comprised of an Active Ingredient Derived from Marijuana to Treat Rare, Severe Forms of Epilepsy

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Español (/news-events/comunicados-de-prensa/la-fda-aprueba-el-primer-medicamento-compuesto-por-un-ingrediente-activo-derivado-de-la-marihuana)

The U.S. Food and Drug Administration today approved Epidiolex (cannabidiol) [CBD] oral solution for the treatment of seizures associated with two rare and severe forms of epilepsy, Lennox-Gastaut syndrome and Dravet syndrome, in patients two years of age and older. This is the first FDA-approved drug that contains a purified drug substance derived from marijuana. It is also the first FDA approval of a drug for the treatment of patients with Dravet syndrome.

CBD is a chemical component of the Cannabis sativa plant, more commonly known as marijuana. 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 marijuana.

"This approval serves as a reminder that advancing sound development programs that properly evaluate active ingredients contained in marijuana can lead to important medical therapies. And, the FDA is committed to this kind of careful scientific research and drug development," said FDA Commissioner Scott Gottlieb, M.D. "Controlled clinical trials testing the safety and efficacy of a drug, along with careful review through the FDA's drug approval process, is the most appropriate way to bring marijuana-derived treatments to patients. Because of the adequate and well-controlled clinical studies that supported this approval, prescribers can have confidence in the drug's uniform strength and consistent delivery that support appropriate dosing needed for treating patients with these complex and serious epilepsy syndromes. We'll continue to support rigorous scientific research on the potential medical uses of marijuana-derived products and work with product developers who are interested in bringing patients safe and effective, high quality products. But, at the same time, we are prepared to take action when we see the illegal marketing of CBD-containing products with serious, unproven medical claims. Marketing unapproved products, with uncertain dosages and formulations can keep patients from accessing appropriate, recognized therapies to treat serious and even fatal diseases."

Dravet syndrome is a rare genetic condition that appears during the first year of life with frequent fever-related seizures (febrile seizures). Later, other types of seizures typically arise, including myoclonic seizures (involuntary muscle spasms). Additionally, status epilepticus, a potentially life-threatening state of continuous seizure activity requiring emergency medical care, may occur. Children with Dravet syndrome typically experience poor development of language and motor skills, hyperactivity and difficulty relating to others.

Lennox-Gastaut syndrome begins in childhood. It is characterized by multiple types of seizures. People with Lennox-Gastaut syndrome begin having frequent seizures in early childhood, usually between ages 3 and 5. More than three-quarters of affected individuals have tonic seizures, which cause the muscles to contract uncontrollably. Almost all children with Lennox-Gastaut syndrome develop learning problems and intellectual disability. Many also have delayed development of motor skills such as sitting and crawling. Most people with Lennox-Gastaut syndrome require help with usual activities of daily living.

"The difficult-to-control seizures that patients with Dravet syndrome and Lennox-Gastaut syndrome experience have a profound impact on these patients' quality of life," said Billy Dunn, M.D., director of the Division of Neurology Products in the FDA's Center for Drug Evaluation and Research. "In addition to another important treatment option for Lennox-Gastaut patients, this first-ever approval of a drug specifically for Dravet patients will provide a significant and needed improvement in the therapeutic approach to caring for people with this condition."

Epidiolex's effectiveness was studied in three randomized, double-blind, placebo-controlled clinical trials involving 516 patients with either Lennox-Gastaut syndrome or Dravet syndrome. Epidiolex, taken along with other medications, was shown to be effective in reducing the frequency of seizures when compared with placebo.

The most common side effects that occurred in Epidiolex-treated patients in the clinical trials were: sleepiness, sedation and lethargy; elevated liver enzymes; decreased appetite; diarrhea; rash; fatigue, malaise and weakness; insomnia, sleep disorder and poor quality sleep; and infections.

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 treat epilepsy, the most serious risks include thoughts about suicide, attempts to commit suicide, feelings of agitation, new or worsening depression, aggression and panic attacks. Epidiolex also caused liver injury, generally mild, but raising the possibility of rare, but more severe injury. More severe liver injury can cause nausea, vomiting, abdominal pain, fatigue, anorexia, jaundice and/or dark urine.

Under the Controlled Substances Act (CSA), CBD is currently a Schedule I substance because it is a chemical component of the cannabis plant. In support of this application, the company conducted nonclinical and clinical studies to assess the abuse potential of CBD.

The FDA prepares and transmits, through the U.S. Department of Health and Human Services, a medical and scientific analysis of substances subject to scheduling, like CBD, and provides recommendations to the Drug Enforcement Administration (DEA) regarding controls under the CSA. DEA is required to make a scheduling determination.

The FDA granted Priority Review (/patients/fast-track-breakthrough-therapy-accelerated-approval-and-priority-review/priority-review) designation for this application. Fast-Track (/patients/fast-track-breakthrough-therapy-accelerated-approval-and-priority-review/fast-track) designation was granted for Dravet syndrome. Orphan Drug (/designating-orphan-product-drug-and-biological-products) designation was granted for both the Dravet syndrome and Lennox-Gastaut syndrome indications.

The FDA granted approval of Epidiolex to GW Research Ltd.

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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Related Information

- FDA and Marijuana (/news-events/public-health-focus/fda-and-marijuana)
- NIH: Lennox-Gastaut Syndrome Information Page (https://www.ninds.nih.gov/Disorders/All-Disorders/Lennox-Gastaut-Syndrome-Information-Page)

- NIH: Dravet Syndrome Information Page (https://www.ninds.nih.gov/Disorders/All-Disorders/Dravet-Syndrome-Information-Page)
- NIH: Marijuana as Medicine (https://www.drugabuse.gov/publications/drugfacts/marijuana-medicine)
- FDA: Approved Drug Questions and Answers (/drugs/information-consumers-drugs/approved-drugs-questions-and-answers)

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