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Epidiolex (cannabidiol) Oral Solution
Clinical Update

Clinical Update: FDA approves Epidiolex (cannabidiol) oral solution to treat seizures associated with tuberous sclerosis complex.
FDA approval date: July 31, 2020

Epidiolex (cannabidiol) is a prescription medicine used to treat seizures in people with Lennox-Gastaut syndrome, Dravet syndrome, or tuberous sclerosis complex for patients at least 1 year old. It is 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.

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 ($\geqslant$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.