Epidiolex® (cannabidiol)

Medicaid Testimony

- Epidiolex® (cannabidiol) is the first and only FDA-approved, prescription cannabidiol indicated for the treatment of seizures associated with Lennox-Gastaut syndrome, Dravet syndrome or Tuberous sclerosis complex in patients 1 year of age and older. The recommended maintenance dose for LGS and Dravet is 10mg/kg/day to 20mg/kg/day and the maintenance dose for TSC is 25mg/kg/day.
- Cannabidiol, the active ingredient in Epidiolex, is highly purified and structurally distinct from other antiepileptic drugs. Although the exact mechanisms of action are unknown, it does not appear to exert its anticonvulsant effects through interaction with cannabinoid receptors. In contrast to THC, it does not have psychoactive or euphoric properties.
- The US DEA confirmed that EPIDIOLEX is no longer subject to the Controlled Substances Act (CSA) and is thus no longer a scheduled substance on the federal level. EPIDIOLEX has also been descheduled in West Virginia.
- Dravet syndrome and Lennox-Gastaut syndrome (LGS) are rare, intractable and severe forms of epilepsy with childhood onset and persistence into adulthood. Patients with LGS or Dravet not only have a high frequency of seizures, but also have developmental and physical disabilities, and high morbidity and mortality.
- The efficacy and safety profile of Epidiolex for the treatment of seizures associated with LGS and Dravet were evaluated in three randomized, double-blind, placebo-controlled, multicenter 14-week trials, where Epidiolex or placebo were added to the patient's current anti-epileptic drugs.
- Epidiolex achieved its primary endpoint of statistically significant median percent change in convulsive and/or drop seizure frequency from baseline in these three studies vs placebo. The treatment effect was established during the first 4 weeks of initiating treatment and was sustained throughout the study period. Results showed 39-44% reduction in median monthly convulsive and/or drop frequency over baseline across all three trials.
- In addition, the secondary endpoint of the proportion of 50% responders was greater among patients receiving Epidiolex compared with placebo across all three trials.
- Tuberous sclerosis complex (TSC) is a highly variable genetic disorder that is characterized by the formation of benign hamartomas in virtually every organ of the body. Patients with TSC commonly experience treatment-resistant epilepsy that can begin in infancy and persist throughout life, with multiple seizure-types. TSC may be associated with highly variable, multi-organ manifestations, neuropsychiatric disorders, intellectual deficits, and significantly increased resource utilization compared with healthy controls.
- The efficacy and safety of add-on Epidiolex for the treatment of seizures associated with TSC was evaluated in a 16-week, randomized, double-blind, placebo-controlled, multicenter trial. Doses of 25 mg/kg/day and 50 mg/kg/day equally and significantly reduced seizures (49% and 48% mean reduction, respectively, compared to baseline) vs. placebo (27% reduction) in the intention to treat analysis (p=.0009, p= 0.018, respectively). While the TSC clinical trial included a 50mg/kg/day arm, greater efficacy was not observed compared with 25mg/kg/day; however, a greater incidence of AEs was observed. Thus, we did not seek approval for this dose.
- As described in the USPI, the most common adverse reactions that occurred in Epidiolex-treated patients with an incidence at least 10% and greater than placebo for those with LGS and Dravet were somnolence, decreased appetite, diarrhea, transaminase elevations, fatigue, malaise, asthenia, rash, insomnia, sleep disorder, poor-quality sleep and infections; those with TSC were diarrhea, transaminase elevations, decreased appetite, somnolence, pyrexia, and vomiting
- Details regarding the contraindication for hypersensitivity and warnings and precautions for hepatocellular injury, somnolence/sedation, suicidal behaviour/ideation, and withdrawal of antiepileptic drugs are provided in the Epidiolex full Prescribing Information.
- Nationally, more than 98% of prescriptions for Epidiolex have been written by Neurology Specialists.
- In Fee For Service Medicaid, prior authorization approval rates exceed 84%. Therefore, please consider leveraging technology, and automating the Prior Authorization process with an electronic look-back for appropriate therapies, thus reducing the burden of the prior authorization process for providers and patients.
- In summary, Epidiolex has been demonstrated as effective for the treatment of seizures associated with Lennox-Gastaut, Dravet Syndrome or Tuberous sclerosis complex in patients 1 year of age and older with a well characterized safety profile.
- Thank you for your consideration.