NYSDOH Epidiolex® Expanded Access Study
Appendix C

Subject Selection *

Inclusion Criteria

Patients who will be selected for this study will meet the following criteria:

1. Not eligible for or enrolled in current GW double-blind clinical studies
2. Ages between 1 year old and 21 years old, male or female, at week 1 (at time informed consent is signed)
3. Must be a resident of New York State
4. Documentation of a diagnosis of drug resistant epilepsy as evidenced by medical records, genetic testing, and/or the following clinical features (must have all 3 of the following):
    a. Patient should have history of a trial of at least four drugs, including one trial of a combination of two concomitant drugs, without successful seizure control. Vagal nerve stimulation (VNS), responsive neurostimulation deep brain stimulation (RNS), or the ketogenic diet can be considered equivalent to a drug trial. Exception to this is a patient who has a confirmed diagnosis of a drug resistant epilepsy syndrome such as Dravet syndrome or Lennox-Gastaut syndrome
    b. An available video-EEG characterization of seizures
    c. Must report at least 8 countable complex partial seizures, atonic, tonic, or tonic-clonic seizures per month. Non-countable seizures include absence and myoclonic
5. Blood work (LFT, BUN, creatinine, CBC, electrolytes and AED levels) completed prior to study entry and assessed by PI prior to first medication administration
6. Between 1-4 baseline AEDs at stable doses for a minimum for 4 weeks prior to enrollment. Vagus nerve stimulator, ketogenic diet, and modified Atkins diet do not count towards this limit
7. Vagus nerve stimulator must be on stable settings for a minimum of 3 months
8. If on ketogenic diet, must be on stable ratio for a minimum of 3 months

Exclusion Criteria

Patients cannot be included in the study if:

1. Eligible for or enrolled in current GW double-blind clinical studies
2. Epilepsies associated with neurodegenerative diseases include neuronal ceroidolipofuscinosis, progressive myoclonus epilepsies, Rasmussen encephalitis, and tumors
3. Epilepsies associated with an inborn error of metabolism, including mitochondrial disorders
4. Less than 8 countable complex partial seizures, atonic, tonic, or tonic-clonic seizures per month. Non-countable seizures include absence and myoclonic
5. Felbatol (felbamate) initiated within the past 12 months
6. LFTs at greater than twice the upper limit of normal
7. Allergy to sesame oil
8. Subjects who plan on travelling internationally while on the study
9. Use of any cannabis-related product in the past month
10. She is pregnant or breastfeeding
11. The patient is of child-bearing potential and is unwilling or unable to commit to using highly effective contraception (e.g. double barrier) for the duration of treatment with Epidiolex

*Modified from criteria kindly provided by Orrin Devinsky, MD, NYU Langone Medical Center