NYSDOH Epidiolex® Expanded Access Study

Appendix B

Study Summary*

Title	A Phase I, Observational Study of Cannabidiol as a Compassionate Use Treatment for Drug Resistant Epilepsies
Short Title	Cannabidiol for Drug Resistant Epilepsies
Funding Sponsor	New York State Department of Health
Phase	Phase 1
Methodology	Observational, Expanded Access study
Study Duration	Procedures and Data collection will last 1 year. Treatment will be provided indefinitely until it is terminated by the Principal Investigator or the FDA or until the FDA approves cannabidiol for drug resistant epilepsy or until the manufacturer no longer supplies the drug.
Study Center(s)	NYU Langone Medical Center Comprehensive Epilepsy Center, University of Rochester Medical Center, Montefiore Medical Center, Mt. Sinai-Beth Israel Comprehensive Epilepsy Center, U. of Buffalo - Women and Children's Hospital of Buffalo
Objectives	To establish optimal dose of cannabidiol for seizure reduction and to provide until it is FDA approved.
Number of Subjects	100 total
Number of clinic visits	7
Laboratory Monitoring	EKG, LFT, BUN, creatinine, CBC, chemistry and AED levels
Diagnosis and Main Inclusion Criteria	Patients with drug resistant childhood onset epilepsy between the ages of 1 and 21 years old male or female at the start of the study. Must 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, RNS deep brain stimulation, or the ketogenic diet can be considered equivalent to a drug trial, OR Must have a confirmed diagnosis of a drug resistant epilepsy syndrome such as Dravet syndrome or Lennox Gastaut syndrome (and not eligible for GW sponsored clinical trials). Must be on a currently stable regimen of 1-4 AEDs
Study Product, Dose, Route, Regimen	Pure Cannabidiol (oral solution 100mg/ml) Initial dose: 5 mg/kg/day orally in 2 equally divided doses Titration: Increased by 5 mg/kg/day at each in-person visit as tolerated over 12 months (up to max dose of 25 mg/kg)
Duration of administration	Indefinitely until study is terminated or until FDA approved.
Reference therapy	None
Statistical Methodology	The primary aims of this study are to achieve seizure control with cannabidiol in children and young adults with drug resistant epilepsies. Therefore, there will be no formal statistical methodology but rather descriptive statistics to quantify the frequency and severity of adverse effects that are reported for the various dosages that are employed.

* May be slightly different at each site to satisfy Institutional Review Board requirements.