NEW YORK STATE OF OPPORTUNITY. Department of Health

ANDREW M. CUOMO
Governor

HOWARD A. ZUCKER, M.D., J.D.
Commissioner

SALLY DRESLIN, M.S., R.N.
Executive Deputy Commissioner

January 20, 2016

Dear Doctor:

The New York State Department of Health (Department) is sponsoring Investigational New Drug studies (IND) using Epidiolex®, a pharmaceutical grade of cannabidiol produced by GW Pharmaceuticals (GW) for treatment resistant epilepsy. The studies are regulated under the FDA's Expanded Access (compassionate use) Program and will be conducted at five major epilepsy centers in New York State (NYS) (Appendix A). A study summary is included as Appendix B. Children and young adults in NYS with treatment resistant epilepsy who meet the inclusion and exclusion criteria (see Appendix C) may be eligible for one of these studies. Although not Food and Drug Administration (FDA)-licensed, Epidiolex® has previously been used in compassionate use cases. Data obtained in the Department's observational studies will contribute to the body of knowledge about the long term tolerability and safety of this drug in children and young adults with epilepsy.

These studies are separate from the phase 2 and 3 controlled clinical trials being conducted by GW to study the use of Epidiolex® in children with Lennox-Gastaut or Dravet syndrome at the same five epilepsy centers. Patients who are eligible for the GW clinical trials will not be considered for the Department's-sponsored studies (Appendix D).

If you are the primary epilepsy health care provider for patients who you believe may benefit from participating at one of the five sites conducting these Expanded Access Investigational New Drug studies of Epidiolex®, please evaluate their eligibility and refer them, if eligible, to one of the sites as detailed below. Each patient can be referred to only one of the five sites. Patients are not able to change study sites once selected. The contact information for the principal investigators to whom to send patient referrals for the Department's-sponsored studies are listed in Appendix A. Patients or patient families should not attempt to contact the study sites directly.

The Department's-sponsored Expanded Access Studies using Epidiolex® will enroll a total of 100 patients in NYS between the ages of 1 and 21 years. Patients must be able to travel to the clinical trial site for scheduled visits (Appendix E). The deadline for submission of referral forms for consideration to participate in one of these studies is March 2, 2016. Participants will be selected by a site-specific random patient selection process from a list of eligible patients for whom referral forms have been submitted by the referral deadline.

A report of an electroencephalographic (EEG) video monitoring study documenting a typical seizure is required and should be submitted with the referral form. For patients selected by the random patient selection process to participate, the principal investigators at the selected site will request a 30 day seizure diary (Appendix F) and may also require submission of a video showing a typical seizure for the patient, filmed by the patient's neurologist or their staff, as part of the review of the patient's eligibility. They will additionally require forwarding of a recent laboratory report with results for a CBC, CMP and AED levels. Initial enrollment of selected patients will begin in February 2016. The studies are anticipated to last one year and will look at the long term safety of the drug. In addition, the study will give NYS physicians clinical experience in using this investigational new drug in severely affected patients. This will benefit

patients more broadly should Epidiolex® be licensed by the FDA. GW has indicated that it will continue to provide Epidiolex to patients chosen to participate in these studies after the studies are completed, prior to its approval by the FDA.

To refer a patient for consideration in the Department's-sponsored studies:

1) Fill out a Department Epidiolex® Expanded Access Study Referral Form (attached);
2) Submit the referral form and report of the EEG video monitoring study directly to the chosen clinical trial site at the address indicated in Appendix A (only one site per patient may be chosen. Do NOT send these forms to the Department);
3) Fill out the de-identified patient Excel spreadsheet (attached) – one spreadsheet is used for all of the patients you are referring – send this spreadsheet to the Department at clinicaltrials@health.ny.gov.;
4) Confirm that an ongoing seizure diary is being maintained or will be immediately initiated for each referred patient;
5) Once the patients are chosen at each of the five clinical sites, you will be contacted by the applicable principal investigator or a member of his or her staff and informed whether your patient has been selected, determined not eligible or wait-listed.

Thank you for considering the above described studies for your patients. Please feel free to forward this announcement to any of your neurology colleagues in NYS who may be interested in referring a patient for consideration in this study. General questions about the Department's-sponsored studies may be sent to clinicaltrials@health.ny.gov.

Sincerely,

Howard Zucker M.D.

Howard A. Zucker, M.D., J.D.
Commissioner of Health