Idaho Expanded Access Program for Epidiolex©

Christine Hahn, MD

10/13/2015

IDAHO DEPARTMENT OF HEALTH & WELFARE

DIVISION OF PUBLIC HEALTH

What is Epidiolex?

- Drug manufactured by GW Pharmaceuticals based in London and currently in FDA-approved clinical trials across the US as a treatment for various pediatric epilepsy syndromes
- Liquid formulation of highly purified plant-derived cannabidiol (CBD) as its active ingredient
- Contains no THC
- Designated as an Orphan Drug by the FDA in the treatment of Dravet and Lennox-Gastaut syndromes, each of which are severe infantile-onset, drug-resistant epilepsy syndromes.
- FDA has granted expanded access INDs to several independent investigators in the U.S. to allow treatment of pediatric epilepsy patients with Epidiolex. These patients suffer from Dravet syndrome, Lennox-Gastaut, and other pediatric epilepsy syndromes.


EPIDOLEX
A 98% CBD EXTRACT FROM CANNABIS
FOR USE IN THE TREATMENT OF EPILEPSY


100
mg/mL
EPIDIOLEX®
(cannabidiol) 100 mg/mL
Oral Solution
CAUTION: New Drug – Limited by Federal
(or United States) Law to
Investigational Use
GW Pharma Ltd
Bottle 1


MEMBERSHIP
JOIN
Join the AAN
Member Benefits
RENEW
Renew Your Membership
Update Your Profile
ENGAGE
Find a Member
Become a Fellow (FAAN)
Committees
Sections
Emerging Leaders Forum
State Societies
ABOUT THE AAN
About the AAN
Press Release
SHARE:
EMBARGOED FOR RELEASE UNTIL 4 PM ET, April 13, 2015
Medical Marijuana Liquid Extract May Bring Hope for Children with Severe Epilepsy
WASHINGTON, DC – A medicinal liquid form of marijuana may show promise as a treatment for children with severe epilepsy that is not responding to other treatments, according to a study released today that will be presented at the American Academy of Neurology's 67th Annual Meeting in Washington, DC, April 18 to 25, 2015.
The study involved 213 people, ranging from toddlers to adults, with a median age of 11 who had severe epilepsy that did not respond to other treatments. Participants had Dravet syndrome and Lennox-Gastaut syndrome, epilepsy types that can lead to intellectual disability and lifelong seizures, as well as 10 other types of severe epilepsy.
The participants were given the drug cannabidiol, a component of marijuana that does not include the psychoactive part of the plant that creates a "high." The drug is a liquid taken daily by mouth. Participants all knew they were receiving the drug in the open-label study, which was designed to determine whether the drug was safe and tolerated well.
AMERICAN ACADEMY OF NEUROLOGY
What are you looking for?
MEMBER LOG IN
Email or 6-digit Member ID
Password
Remember me
Forgot password?
LOG IN
Not a member yet? Join us now!
QUICK LINKS
Find a Member
Renew Your Membership
Update Your Profile
Track Your CME
Join the AAN
The AAN Store

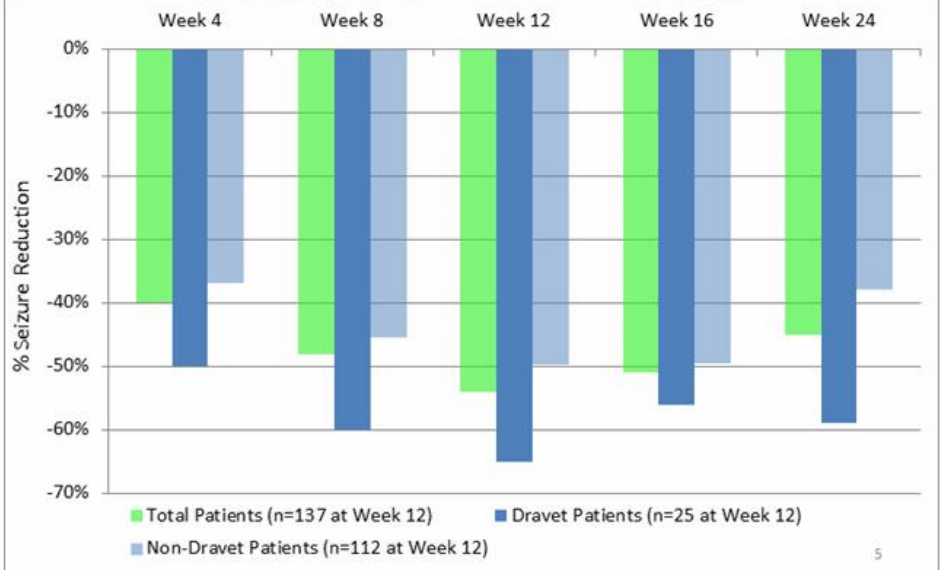
Initial study results– U.K.

- British study results presented American Academy of Neurology April 18-25 meeting
- 213 people, ranging from toddlers to adults, with a median age of 11 who had severe epilepsy that did not respond to other treatments. Participants had Dravet syndrome and Lennox-Gastaut syndrome, epilepsy types that can lead to intellectual disability and lifelong seizures, as well as 10 other types of severe epilepsy.
- For the 137 people who completed the 12-week study, the number of seizures decreased by an average of 54% from the beginning of the study to the end.

GW Pharma- Global Healthcare Conference 2015
Thursday, September 17, 2015, 10:25 am EDT

Slide | Search Slides | Downloads

American Academy of Neurology – April 15
Median % Reduction in Total Seizures



00:07:40/00:37:32

Slide 5 of 8

Home

Powered by NASDAQ OMX | Help

Idaho involvement

- Clinician in Idaho already participating in clinical trial of Epidiolex© among adults with epilepsy
- This clinician agreed to also apply for Expanded Access approval for children

The Office of the Governor

Executive Department
State of Idaho

EXECUTIVE DEPARTMENT
STATE OF IDAHO
BOISE

State Capitol
Boise

EXECUTIVE ORDER NO. 2015-03

AUTHORIZING THE DEPARTMENT OF HEALTH AND WELFARE TO IMPLEMENT A FDA-APPROVED EXPANDED ACCESS PROGRAM FOR TREATMENT-RESISTANT EPILEPSY IN CHILDREN

WHEREAS, Idaho's citizens with severe or life-threatening diseases or conditions may not be able to access critical medications that are still in clinical trials; and

WHEREAS, the U.S. Food and Drug Administration (FDA) has established Expanded Access Programs to allow limited, supervised access to such medications; and

WHEREAS, the FDA has approved an Expanded Access Program for Epidiolex®, a drug being evaluated for treatment-resistant epilepsy; and

WHEREAS, it is estimated that eight people per 1,000 have active epilepsy; and

WHEREAS, there are children in Idaho with treatment-resistant epilepsy who may benefit from Epidiolex®; and

WHEREAS, the Department of Health and Welfare operates to improve the health status of Idahoans, increase the safety and self-sufficiency of individuals and families, and enhance the delivery of health and human services;

NOW, THEREFORE, I, C.L. "BUTCH" OTTER, Governor of the State of Idaho, by virtue of the authority vested in me by the Constitution and laws of the State of Idaho, do hereby order as follows:

1. *The Department of Health and Welfare shall investigate the need for, and implement if appropriate, as determined by the Department, a FDA-approved Expanded Access Program for Epidiolex®;*
2. *Further, as part of the investigation, the Department shall estimate the scope of the need in Idaho for this program, and shall determine whether appropriate medical supervision is available that allows safe and effective implementation of such a program;*
3. *If implemented, the Department shall investigate and monitor long-term solutions, such as licensure of the medication, that may reduce or eliminate the need for the program in the future; and*
4. *The Department shall track funding utilized for the program and may accept private contributions, federal funds, funds from other public agencies or any other source for the purpose of implementing this study.*

IN WITNESS WHEREOF, I have hereunto set my hand and caused to be affixed the Great Seal of the State of Idaho at the Capitol in Boise on this 16th day of April, in the year of our Lord two thousand and fifteen, and of the independence of the United States of America the two hundred thirty-ninth and of the Statehood of Idaho the one hundred twenty-fifth.

C.L. "BUTCH" OTTER
GOVERNOR

LAWERENCE DENNEY
SECRETARY OF STATE

Progress

- Contract established between IDHW and clinician
- Investigational New Drug (IND) protocol submitted and approved by the FDA
- Memorandum of Understanding signed between IDHW and GW Pharmaceuticals
- Institutional Review Board at IDHW approved the IRB application with minor changes
- Clinician applied 10/7 to USDOJ to request modification of his DEA Registration to include the FDA approved protocol for an IND exemption for the clinical study of Epidiolex©, as a supplemental protocol

IDAHO DEPARTMENT OF
HEALTH & WELFARE

C.L. "BUTCH" OTTER – Governor
RICHARD M. ARMSTRONG – Director

DIVISION OF PUBLIC HEALTH
450 West State Street, 4th Floor
PO Box 83720
Boise, ID 83720-0036
PHONE 208-334-5535
FAX 208-332-7307

Dear Dr. Smith, 8/10/2015

As you may be aware, in April 2015, the Governor of Idaho authorized the establishment of a Food and Drug Administration (FDA)-approved Epidiolex® Expanded Access Program for treatment-resistant epilepsy in Idaho children. Executive Order 2015-03 directs the Department of Health and Welfare to implement and oversee the program.

Expanded access (previously known as "compassionate use") provides a pathway for patients to gain access to investigational drugs, biologics and medical devices for serious diseases or conditions. The FDA is allowing GW Pharmaceuticals, the manufacturer of the purified cannabidiol (CBD) oil Epidiolex®, to initiate Expanded Access Programs in order to facilitate access to Epidiolex®, an investigational drug under evaluation for patients with severe epilepsy not responding to current treatments and who lack therapeutic alternatives.

The program will screen and offer treatment to eligible Idaho children aged 0-18 years of age with intractable epilepsy, and gather preliminary information for phased trials that will determine whether Epidiolex® can reduce epileptic seizures more effectively than standard medications. Idaho has received approval from FDA and GW Pharmaceuticals to include as many as 25 children. Children, once enrolled, would be allowed to continue on this medication until it is available as a prescription medication. This is not a clinical trial, and no placebo arm will be taking place. Note that the Epidiolex® Expanded Access Program is in addition to a separate, nationwide, clinical trial that is occurring this year, including in Idaho.

At present, the only site approved to implement the Epidiolex® Expanded Access Program in Idaho is at Consultants in Epilepsy & Neurology PLLC, the private practice of Robert T. Wechsler, M.D., Ph.D., located in Boise.

If you have pediatric patients with refractory epilepsy who are not responsive to conventional treatment, and not utilizing any form of cannabinoids presently, we encourage you to consider referring them for evaluation for this program. Please feel free to contact Dr. Wechsler at (208) 275-8585 or myself with any further questions. In addition, information about the EAP will be maintained at the IDHW website, at www.eap.dhw.idaho.gov. My contact information is listed below.

Sincerely,

Christine Hahn, MD

Christine Hahn, MD
Medical Director, Division of Public Health
208-334-5939

Current Status/Next Steps

- As soon as approval is received from the USDOJ, screening and enrollment should be able to begin
- The number of children allowed onto the program is controlled by FDA and GW to allow compassionate use of the medication but not hinder enrollment into clinical trials– Idaho will be allowed to enroll 25 children only
- Medication is provided free by GW to enrolled children, but costs for clinical monitoring will be paid for by IDHW

QUESTIONS?