Georgia Cannabidiol Study  
Frequently Asked Questions

1. Why was the Georgia Cannabidiol Study initiated?

In January 2014, Representative Allen Peake visited with Haleigh Cox, a 4-year-old girl with severe seizure disorder. Representative Peake said, “I am an unlikely champion for this cause.... But I had a visit with Haleigh Cox, the daughter of a constituent of mine. The result of seeing the pain and suffering she goes through, having 100 seizures a day, and seeing a potential remedy through cannabidiol treatment, I was compelled to move to action.”

Representative Peake drafted a limited-scope bill that would legalize cannabidiol oil in the state of Georgia to help children with uncontrolled seizures. In March 2014, the bill, House Bill 885, 2014 session, was not passed and prompted Governor Nathan Deal to take action.

Georgia Governor Nathan Deal requested Dr. Michael Diamond, Vice President of Clinical and Translational Sciences at Augusta University, to initiate a “fact finding mission” to:

- Identify options for gaining access to cannabidiol (CBD) for research studies.
- Determine the steps necessary for initiating a study with cannabidiol for medication resistant epilepsy.

Governor Deal in partnership with Rep. Peake and the University System of Georgia announced that a state initiative, led by Augusta University, would be initiated, to conduct a scientific study for treating children with medication resistant epilepsy with CBD.

2. What options were considered as sources for Cannabidiol?

There were four options evaluated as possible sources of cannabidiol for the State of Georgia Cannabidiol Study. The pros and cons of each source were evaluated to determine what source could adequately and safely provide access to Cannabidiol for residents of Georgia. The four sources were:

a) Medical Marijuana
b) Artisanal Cannabidiol (CBD)
c) Cannabidiol (CBD) from Federally Funded Institutions (NIDA)
d) Pharmaceutical Grade Cannabidiol (CBD)

3. Was Medical Marijuana evaluated as a source for the Georgia Cannabidiol Study?

“Medical Marijuana,” in the way the term is commonly utilized, is not the same as Cannabidiol (CBD). Medical Marijuana is normally a dried form of the marijuana plant, Cannabis sativa, which is administered by smoke, vapours, or is added to foods, or drinks. Medical marijuana exposes the individual to all components of the marijuana plant, including cannabidiol (CBD), Tetrahydrocannabinol (THC), as well as many other compounds. There are many different varieties of cannabis plants used for medical marijuana and the strain of the plant provides variations in amount of CBD, THC, and other compounds that can be absorbed by human. For some medical conditions, medical marijuana has been reported to be very effective in pain and/or symptom management.
However, for children with epilepsy, exposure to THC, as well as other compounds often found in marijuana, may be detrimental to their fragile and developing neurological system. Additionally, the amount of CBD present in medical marijuana and the amount that could be absorbed through the usual methods of medical marijuana administration (i.e. smoke, food, etc.) is very difficult to determine. Finally, the use of medical marijuana is currently prohibited by federal and state law in Georgia. For all of these reasons medical marijuana was not deemed to be an effective source for providing CBD to pediatric epilepsy patients.

4. Was Artisanal Cannabidiol (CBD) evaluated as a source for the Georgia Study?

Cannabidiol is a specific part of Cannabis sativa, the marijuana plant. In recent years, there has been an increase in the availability of “artisnaal” cannabidiol as many marijuana dispensaries across the United States have recognized the potential benefits of providing Cannabidiol (CBD) to consumers and have begun extracting and purifying Cannabidiol into a liquid or oil form. Within the United States there are many dispensaries claiming to provide pure cannabidiol, but the variations in production, adherence to standards in the extraction process, and quality assurance processes may vary significantly from supplier to supplier and producer to producer. Dispensaries, although licensed in some States, do not have the appropriate Federal certifications to allow the transport across State lines, to dispense as part of a federally approved clinical study, nor do they always meet production certifications that are required for good manufacturing practice certifications. Resultantly, the use of Artisanal Cannabidiol in the State of Georgia Cannabidiol program was not deemed as an effective or legal approach for providing CBD to pediatric epilepsy patients in Georgia.

5. Were sources of Cannabidiol (CBD) from Federally Funded Institutions (NIDA) evaluated as a source for the Georgia Study?

The National Institute on Drug Abuse (NIDA) supports a drug supply program, which provides controlled substances (including research-grade marijuana) to researchers for scientific purposes. NIDA has contracts with the University of Mississippi to grow marijuana for use in research studies. The marijuana is grown, harvested, stored, and made into purified elements of marijuana to use for research.¹

As a part of the State of Georgia’s “fact finding mission” to identify sources of Cannabidiol (CBD), The University of Mississippi was contacted to determine if their supply could support the State of Georgia’s initiative. It was determined that Cannabidiol for use in this study was not currently available.

6. Were there any Pharmaceutical Grade sources of Cannabidiol for the Georgia Study?

Augusta University contacted the Food and Drug Administration (FDA) in May 2014. Later, it was determined that GW Pharmaceuticals had been granted “orphan drug designation” for treatment of Dravet Syndrome (November 2013) and Lennox-Gastaut Syndrome with Epidiolex®, a pharmaceutical grade Cannabidiol (CBD) medicinal product. Since GW Pharmaceuticals had a pharmaceutical grade product which had been approved by the FDA for use in clinical trials, the decision was made to actively engage GW Pharmaceuticals in discussions about a State supported Cannabidiol program.

¹“This paper expresses the views of Augusta University. While we routinely coordinate with the University System of Georgia, we cannot officially speak on their behalf.”
7. Why a “Memorandum of Understanding” (MOU) with GW Pharmaceuticals?

GW Pharmaceuticals was approached by Augusta University in May 2014 to determine their interest and ability to work with the State of Georgia to help develop a cannabidiol study for pediatric epilepsy. Based upon these discussions, GW Pharmaceuticals, the State of Georgia, and Augusta University Research Institute at Augusta University, agreed to a memorandum of understanding (MOU) to design a research program focused on developing rigorous data to inform and expand the scientific community’s understanding of potential treatments for children with medication-resistant epilepsies.

It was decided that GW Pharmaceuticals was the key partner for the State of Georgia in proceeding with a State Cannabidiol program, for many reasons, including their extensive international network of prominent scientists, and their success in partnering with more than 29 Universities around the world, as well with other pharmaceutical companies for various other ventures.

8. Why was Augusta University (AU) asked to be the Managing Institution for the study?

Augusta University (AU) is Georgia’s public Medical Research University and home of the Medical College of Georgia (MCG). AU was renamed from Georgia Regent’s University (GRU) in January 2015. GRU was formed, in January 2013, by the merger of 2 universities, Georgia Health Sciences University and Augusta State University. This new comprehensive university consists of 9 Colleges and the Georgia Regents Health System. AU through the Medical College of Georgia and the Children’s Hospital of Georgia provides the expertise in clinical trial design, the support and infrastructure of an academic health sciences research institution, combined with world class pediatric epilepsy and neurology care.

9. Are there other pharmaceutical grade products available?

To our knowledge, Epidiolex®, by GW Pharmaceuticals, is the only Cannabidiol, currently being used as part of a FDA trial for medication resistant epilepsy. Epidiolex® is a liquid formulation of highly purified Cannabidiol (CBD) extract and is an investigational drug for the treatment for various orphan pediatric epilepsy syndromes. Orphan Drug Designation has been granted by the FDA in the treatment of Dravet Syndrome and in Lennox Gastaut Syndrome. Epidiolex® has not been approved for use by the FDA except as a part of clinical trials or expanded access treatment programs.

10. What is a Clinical Trial?

Clinical trials are research studies that explore whether a medical strategy, treatment, or device is safe and effective for humans. These studies also may show which medical approaches work best for certain illnesses or groups of people. Clinical trials produce the best data available for health care decision making.

The purpose of clinical trials is research, so the studies follow strict scientific standards. These standards protect patients and help produce reliable study results. For safety purposes, clinical trials start with small groups of patients to find out whether a new approach causes any harm. In later phases of clinical trials, researchers learn more about the new approach’s risks and benefits. A clinical trial may find that a new strategy, treatment, or device

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improves patient outcomes;
- offers no benefit; or
- causes unexpected harm
All of these results are important because they advance medical knowledge and help improve patient care.

11. What Is an Expanded Access / Clinical Treatment Study?

Expanded Access Protocols, are sometimes referred to as "compassionate use" protocols. In these studies, the investigational new drug is used to treat patients, outside of a clinical trial, but the following must apply:

1. The patient or patients to be treated have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
2. The potential patient benefit justifies the potential risks of the treatment use and those potential risks are not unreasonable in the context of the disease or condition to be treated; and
3. Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.

The Georgia Cannabidiol Study is an Intermediate Size Expanded Access Treatment protocol. GW Pharmaceuticals, is making their investigational new drug, Epidiolex®, available, under a FDA approved application/protocol, to treat patients with medication resistant epilepsy, who cannot participate in a controlled clinical trial.

12. What exactly is involved in the Georgia Cannabidiol Studies?

The Georgia Cannabidiol studies provide two opportunities for Georgia’s children ages 1 through 18, who have medication resistant epilepsy, to gain access to cannabidiol. These studies include the following:

- Expanded Access Treatment Protocol for 2 patients
  - Under the Principal Investigator, Dr. Yong Park, two children with medication resistant epilepsy, will receive treatment of Epidiolex® at Augusta University for a minimum of one year.
- Multi-Center, Open Label, Expanded Access Protocol
  - This study is approved to treat 50 children (ages 1 through 18) and is planned to be conducted at four sites across Georgia. Sites include Augusta University, in Augusta, GA, two sites in Atlanta, and one site in Savannah.
  - Patients enrolled in this study are expected to receive treatment of Epidiolex® for a minimum of one year.

13. What approvals were required to start the Georgia Cannabidiol Studies?

All research requires regulatory approval and oversight. Specifically, the conduct of these studies are subject to the approval and oversight by the Food and Drug Administration (FDA), the Drug Enforcement Agency (DEA), the Georgia Drugs and Narcotics Agency, and Institutional Review Board.

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Boards (IRB). The FDA through data collected from GW Pharmaceuticals’ randomized control trials, is the governing agency, which may approve Epidiolex® for market use. Additional details about the role of the FDA plays in the regulation of researching marijuana for potential medical uses in the United States can be found at “Mixed Signals: The Administration’s Policy on Marijuana –Part Four – the Health Effects and Science.”

14. How much this study cost and what does this cost cover?

It is predicated that $4.8 million dollars is needed to cover the costs of the Georgia Cannabidiol Studies. This funding includes the research program costs for 1 year of treatment for 52 patients including items such as the following:

- Coordinating center personnel for services such as project managers, regulatory staff, statistical personnel, scientific writing and protocol development.
- Research data collection devices and programs to ensure that the highest integrity of data is collected and maintained.
- Clinical Site Specific Costs
  - Preparing and filing regulatory approvals such as Institutional Review Boards (to ensure human subject protection), DEA approvals, FDA applications, etc., at each clinical site.
  - Research procedures (e.g. safety and efficacy measurements not covered by insurance) as well as biological sample processing and storage for all patients.
  - Research staffing costs (i.e. Investigators and Coordinating Personnel) at all sites.
- Travel and supply costs for each clinical site and the coordinating center.

15. Are the Studies open to Enrollment?

The studies are open for enrollment. For more details about recruiting locations, please call 706-721-3371.

16. What other studies are happening?

It is anticipated that GW Pharmaceuticals will also be providing the opportunity for two clinical sites in Atlanta and Augusta, to enroll patients in a national research study for two orphan indication syndromes - Dravet Syndrome and Lennox-Gastaut Syndrome. These randomized control trials are funded in their entirety by GW Pharmaceuticals and are anticipated to open in the spring of 2015.
REFERENCES

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