

The Senate Caucus on International Narcotics Control

“Cannabidiol: Barriers to Research and Potential Medical Benefits”

Wednesday, June 24, 2015 at 9:30AM

**Testimony from
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Chairman Grassley, Co-Chairman Feinstein, Senator Gillibrand and distinguished members of the Senate Caucus on International Narcotics Control, thank you for allowing me to submit testimony.

I am the Director of Pediatric Epilepsy at the Mount Sinai Health System in New York. The Mount Sinai Health System is an integrated health care system encompassing the Icahn School of Medicine at Mount Sinai and seven hospital campuses in the New York metropolitan area, as well as a large regional ambulatory footprint.

As you may know, epilepsy is the fourth most common neurological disorder in the United States. The annual incidence of new cases of epilepsy varies from about 40 per 100,000 in adults to 150 per 100,000 in children. Of these new cases, 50 to 65 percent become seizure free with medications but, unfortunately, 10 to 20 percent (about 600,000 individuals) become medically intractable and continue to have debilitating, life threatening seizures despite our best attempts at medical or lifestyle treatments.

In my work at Mount Sinai, I have seen this first hand. We work with over 5,000 children and developmentally delayed adults struggling with severe intractable epilepsy. Severe intractable epilepsy is epilepsy that does not respond to two appropriate medications used at the correct doses. Unfortunately, despite our best efforts, this condition does not respond to any available treatment. Our daily goal with patients is to provide treatment in the form of medications, surgery and diet, and to try and reduce the number or severity of seizures but our patients still struggle with life threatening seizures that can cause falls, injuries that include fractures, burns and concussions. Side effects of the medications can also include dizziness, headaches, insomnia, sleepiness, anxiety, tremors, rashes, confusion, and cognitive dysfunction.

For many of the parents, one of the harshest realities is the compounding risk of death and significant injury from these seizures. There is a high incidence (about 50 percent) of associated co-morbidities, including psychiatric illness, mental retardation, cerebral palsy, developmental delays, depression and learning disabilities that comes with this condition. In addition,

individuals with epilepsy are far less likely than those with other chronic diseases to live independently, graduate from high school, be employed, have children and be in a long-term relationship.

Furthermore, we have seen limited progress over the past 40 to 50 years despite an armamentarium of new second and third generation medications to try and stop seizures and allow development of the brain. Patients with epilepsy and their families are desperate to find ways to treat this disease. So are their health care providers. When FDA approved treatments fail, families often turn to alternative treatments, like botanicals, which are less scrutinized and do not undergo rigorous double blind testing.

Cannabis or marijuana is one of those botanicals that some families have turned to. Historically, it has actually been used for centuries to treat a variety of medical disorders. It was first used to treat epilepsy in medieval times and again in the 19th century for infantile spasms (one of the most devastating of childhood epileptic encephalopathies).

However, currently, the research is limited. There are only a limited number of studies with a limited number of participants and they have incomplete data regarding efficacy. We need to be able to run rigorous clinical trials and tests, as quickly as safely possible, so that if it is efficacious we can quickly begin treating these children.

A number of pharmaceutical companies are currently investigating the efficacy of Cannabidiol (CBD), which is one of the ingredients in marijuana, as a treatment for seizures associated with Dravet syndrome and Lennox-Gastaut syndrome. However, while we wait for these results, there is a growing movement amongst families to procure CBD made from hemp oil or obtained from states where medical marijuana is available.

Every day families come to us and say that they are going to obtain CBD and ask us for help in dosing. We obviously decline their requests but we understand their desperation –what parent wouldn't fight to the end to save their child's life. However, as a provider and researcher, we need legal access to this compound to be able to put the proper research protocols into place in order to study this treatment and learn if it is effective and, if so, how to accurately dose.

One of our patients, Morgan, can serve as an example of just how difficult this disease can be and of the extraordinary lengths that these families go to in order to obtain treatment for their children. Morgan began to have seizures soon after birth. In her four years of life, she has been treated with, and failed on, over ten different medications. She has been hospitalized more than twenty times, and undergone hundreds of tests. At the same time, she has been diagnosed with a movement disorder so severe that it is almost impossible for her to sit, eat and sleep. She does not walk and does not speak. She was eventually diagnosed with Dravet's syndrome, a genetic channelopathy that causes intractable seizures, severe intellectual delays and movement disorders.

Her parents have heard promising stories from other families about the possibility of cannaboid for treatment. Her parents are patiently waiting for cannaboid to go through the proper clinical trial testing. Meanwhile, they are trying to avoid using the readily available but less regulated hemp oil preparations because they firmly believe in the scientific method. However, time is running out.

Morgan came to Washington earlier this year with her mom, Kate Hintz, to support Senator Gillibrand's CARERS Act, which would reschedule medical marijuana to a schedule 2 from a schedule one drug, allowing for easier research.

The biggest obstacle facing the clinical trials is that of the schedule one licensing. It is much more difficult to start a clinical trial on a schedule one medication because the current laws make this extraordinarily prohibitive. I feel strongly that Pure CBD products should not be a schedule one substance since this is not marijuana. Pure CBD does not include THC, which is the psychoactive component that produces the "high."

The CARERS Act is not asking for THC (the psycho-active component of marijuana) to be changed from schedule one to a schedule two. This important legislation would change only CBD to a schedule two drug. This bill will allow major medical research centers to perform scientific studies to prove the efficacy and safety of all the available CBD products on the market.

The scientific community would then be able to do double -blinded placebo controlled studies to prove if these CBD compounds will be efficacious for patients with epilepsy and other severe medical disorders. The medical providers will be able to prove for once and for all if all the excitement and hype about CBD is real and will be able to do this with clear science. The medical community will also be able to clarify the safety of dosing and the side effects.

It is our job as health care providers, scientists and community leaders to protect our patients, friends and families with the best scientific data possible. Without the ability to do this research there is no agreed upon dosing schedule and we do not know what the long term side effects are. We need this change in law to prove (or dis-prove) efficacy and safety and allow the scientists, doctors and FDA to do their job by providing thoughtful studies to protect the health of our country.

Thank you to Senators Booker, Gillibrand, and Paul for their commitment to the CARERS Act which will facilitate research on cannabis and help the epilepsy community potentially gain safe, legal access to this treatment option. I also want to thank this Caucus for bringing this important conversation to the forefront. I remain hopeful that we will be able to provide these families with a working treatment they so desperately need and deserve.

Thank you again for allowing me to submit testimony today.