Clinical trial results of medicinal marijuana for treatment in Dravet syndrome

Research findings have just been published from the first randomised, double-blind, placebo controlled trial using medicinal marijuana for the treatment of a form of epilepsy in the prestigious medical journal the New England Journal of Medicine.


This clinical trial involved a large collaboration (known as the Cannabidiol in Dravet Syndrome Study Group) from several countries around the world. One of the study group members was Professor Ingrid Scheffer, an epileptologist (specialist neurologist) from the Florey Institute at Austin Health in Melbourne.

The clinical trial looked at whether cannabidiol (medicinal marijuana) was effective for the treatment of drug-resistant seizures in Dravet syndrome - a complex form of childhood epilepsy as an additional treatment to their current anti-epileptic drugs.

What does the research say about the use of medicinal marijuana for epilepsy?

- Treatment over 14 weeks with cannabidiol (medicinal marijuana) was effective – it significantly reduced the rate of convulsive seizures, but not non-convulsive seizures. Higher rates of adverse events and more withdrawals from the trial were also found following treatment with cannabidiol.
- 43% of patients treated with cannabidiol had at least a 50% reduction in convulsive seizures in comparison to only 27% who had a placebo treatment.
- People's overall condition showed improvement in 62% of patients taking cannabidiol in comparison to only 34% of the placebo group.
- 5% of all patients who took cannabidiol were seizure-free whereas no patients in the placebo group were found to be seizure-free.

Although the results of this randomised clinical trial are very promising, some caution should be applied - cannabidiol should not be used as a standalone treatment for drug-resistant Dravet syndrome. Cannabidiol is a treatment but not a cure for Dravet syndrome which is a rare genetic form of epilepsy.

Future research studies should look at the effectiveness of cannabidiol and any adverse effects over a longer period of treatment.

What is Dravet Syndrome?

Dravet syndrome is a type of epilepsy which starts in childhood around 6 months of age and is caused by a rare genetic mutation in the SCN1A gene. Seizures are triggered by hot temperature or fever, also called febrile seizures, and get progressively worse with age developing into several other different types of seizures.
Seizures experienced in Dravet syndrome are difficult to control as they do not respond well to anti-epileptic drugs, this concept is referred to as being drug-resistant. Children with Dravet syndrome have severe developmental problems and there is also a high rate of death (mortality) which can occur.

What is cannabidiol?

Cannabidiol or CBD is a type of cannabinoid which is one of the main ingredients in the marijuana plant *Cannabis sativa*. There are approximately 100 different cannabinoids present in the plant. One of the most well-known cannabinoids is tetrahydrocannabinol or THC which produces hallucinogenic effects - the ‘high’ associated with marijuana.

Cannabidiol does not produce any hallucinogenic effects, however it is thought to respond to seizure activity, a key symptom of epilepsy.

Medicinal marijuana is mostly made up of cannabidiol with a relatively low amount of THC. Illegal marijuana on the other hand is mostly made up of THC and a low amount of cannabidiol.

Why was the research done?

Multiple anecdotal reports have shown that medicinal marijuana is effective and beneficial for the treatment of seizures in epilepsy. However, there is a lack of high quality (well-controlled) scientific evidence-based studies to support these findings.

To date there have only been 4 small trials using cannabidiol for the treatment of epilepsy but the findings have not been clear. Findings from an open-label trial – a trial where both the researchers and participants know which treatment is being provided - was published last year in the Lancet medical journal but this was not a double-blind randomised clinical trial. Therefore, it was important to know if cannabidiol was effective for drug-resistant types of epilepsy such as Dravet syndrome using high-quality scientific methods.

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