The Queensland Government has established a partnership with GW Pharmaceuticals, a UK-based pharmaceutical company, for medicinal cannabis trials for children with severe treatment-resistant epilepsy.

The first of these trials will be using a pure cannabidiol product, Epidiolex®. Cannabidiol is a non-psychoactive cannabinoid. GW Pharmaceuticals has a number of other cannabis-derived pharmaceutical products in the development pipeline.

**Clinical trial using Epidiolex®**

The agreement with GW Pharmaceuticals allows for a clinical trial for compassionate access for a limited number of eligible children suffering from severe treatment-resistant epilepsy to use the medicine Epidiolex®.

The children will be able to use Epidiolex® as part of their treatment plan under a medically supervised trial that will take place at the Lady Cilento Children’s Hospital (LCCH).

**Key outcomes under the partnership for Queensland**

1. A clinical trial using Epidiolex® for a maximum of 30 children with severe treatment-resistant epilepsy.
2. The establishment of a specialist centre to oversee the clinician-led trials into medicinal cannabis.

**Substances for the trials**

The GW Pharmaceutical product Epidiolex® will be used in the clinical trial. It is a recognised medicine, not a crude cannabis product, and has been manufactured to the highest specification. The product is pharmaceutical grade and made in a GMP (Good Manufacturing Process) accredited facility.

Epidiolex® a liquid formulation of pure plant-derived cannabidiol, or CBD, which is in development for the treatment of a number of rare paediatric epilepsy disorders. Epidiolex® is taken orally and is currently being investigated overseas for the treatment of various rare childhood epilepsy syndromes, including Dravet syndrome and Lennox-Gastaut Syndrome. Epidiolex® has shown positive results in a compassionate use scheme in the United States and in Phase 3 trials in patients with Dravet syndrome and Lennox-Gastaut syndrome.

**Research to date**

A number of trials of the cannabinoid medicine, cannabidiol (Epidiolex®), in children with seizure disorders have shown promising results in certain sub-groups of children with epilepsy, including positive results in two Phase 3 clinical trials in the US and Europe in patients with Dravet syndrome and Lennox-Gastaut syndrome. Other cannabinoids, such as cannabidivaric (CBDV), have shown promise in animal models of epilepsy. Clinical evaluation has shown the possibility that cannabidiol may interact with other anti-epileptic medicines, and the precise nature of these potential interactions is being
investigated in clinical trials. This means that children need to be carefully monitored for side effects and to ensure that their drug levels remain therapeutic and do not enter the toxic or harmful range.

Cannabis-derived medicines do not work for all children with epilepsy and some children do not tolerate the side effects and need to discontinue the treatment. However it is expected that some children will have a reduction in seizures and it is hoped that a smaller number may become seizure free. These trials will help clinicians understand who is most likely to benefit from using these medicines and what side effects they need to watch out for if these medicines become used more frequently.

**Leading the trials**

A team of researchers from Children’s Health Queensland (CHQ) at the LCCH will develop the trials under the leadership of LCCH Director of Paediatric Neurosciences, Dr Geoff Wallace.

**Purpose and Design of the clinical trials**

In the first instance, the purpose of the trials will be determining if Epidiolex® is effective for the treatment of children suffering severe treatment-resistant childhood epilepsy.

The design of this initial trial will be almost the same as that of the compassionate access scheme being undertaken in NSW. This will allow collection of a large amount of data that will be used to inform ongoing trials or development of protocols for the use of this product in Australia.

**Participating in the trials**

Due to the limited quantity of GW Pharmaceuticals’ product Epidiolex® that can be obtained at this time, a maximum of 30 children with severe treatment-resistant epilepsy, no co-existing conditions, and who are under the care of a paediatric neurologist, will be able to take part in the trial.

Not every child who takes part in the trial will obtain benefit, due to the complex nature of treating treatment-resistant epilepsy.

Anyone who thinks they are eligible and is interested in participating in the trial can call 13 HEALTH to register an expression of interest, or talk to your treating doctor.

Families will be advised if they meet the criteria and will be invited to take part in the trial.

Recruitment for the trials is likely to start on 1 November 2016, and pending approval, the trials should start by the end of 2016.

**More information**

Participants will need to meet specific criteria to participate in the trial:

Anyone who thinks they are eligible and is interested in participating in the trial can call 13 HEALTH to register an expression of interest, or talk to your treating doctor.