

Clinical trials for medicinal cannabis to treat epilepsy in children

Children's Health Queensland is currently conducting a Compassionate Access Scheme (CAS) to help test Epidiolex®, the first in a new class of CBD anti-epileptic drugs, as a treatment for children with severe treatment-resistant epilepsy.

About the scheme

An agreement between Queensland Government and UK-based company [GW Pharmaceuticals](http://www.gwpharm.com/Epidiolex.aspx) (<http://www.gwpharm.com/Epidiolex.aspx>) was signed in July 2016 to allow up to 30 Queensland children to access Epidiolex®. The number of participants is restricted by the limited amount of product that can be supplied by the manufacturer.

Children are eligible to apply for the CAS if they:

- are between 2 and 18 years old
- have severe treatment-resistant epilepsy
- are under the care of a paediatric neurologist.

Participants are given Epidiolex® as part of their treatment plan by a consultant neurologist at the [Lady Cilento Children's Hospital](https://www.childrens.health.qld.gov.au/lcch) (<https://www.childrens.health.qld.gov.au/lcch>).

Recruitment for the CAS began in November 2016 and the first participant began receiving Epidiolex® in February 2017.

Research design

The scheme is similar to the [compassionate access scheme currently conducted in New South Wales](https://www.medicinalcannabis.nsw.gov.au/clinical-trials/pediatric-epilepsy-trial) (<https://www.medicinalcannabis.nsw.gov.au/clinical-trials/pediatric-epilepsy-trial>). Both schemes allow the collection of data to inform ongoing trials and protocols for the use of Epidiolex® in Australia.

Not every child who takes part in the scheme will receive benefit due to the complex nature of managing treatment-resistant epilepsy. These medicines may not work for many participants and some may have side effects. All children are carefully monitored by the research team.

Register your interest

If you believe your child is eligible for the scheme:

- talk to your treating doctor
- register an expression of interest by calling the Clinical Research Manager (Neurodevelopmental Disorders) on 07 3069 7405.

You will be advised if your child meets the initial criteria and provided with application details.

If you are not eligible for the CAS, you can still talk to your doctor about whether medicinal cannabis would be a suitable treatment option.

[Apply to use medicinal cannabis products](http://www.qld.gov.au/health/conditions/all/medicinal-cannabis/index.html)

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Epidiolex®

Epidiolex® is a pharmaceutical formulation of purified cannabidiol (CBD), approved for clinical trials by the Therapeutic Goods Administration (TGA).

Epidiolex® is not medical marijuana; it is a liquid formulation of purified CBD that is derived from the cannabis plant. If approved, it would be the first in a new class of CBD anti-epileptic drugs.

Epidiolex® is being studied in large, well-controlled clinical trials with the goal of producing a high-quality, substantial volume of safety and efficacy data to submit to national regulatory agencies such as the TGA. Epidiolex® is manufactured in compliance with [Good Manufacturing Process \(GMP\)](https://www.tga.gov.au/good-manufacturing-practice-overview) (<https://www.tga.gov.au/good-manufacturing-practice-overview>) standards and meets the standards for pharmaceutical products, which ensures consistent formulation and reliable dosing.

Previous research

The efficacy and safety of Epidiolex® is currently being investigated overseas for the treatment of various rare childhood epilepsy syndromes, including Dravet Syndrome and Lennox-Gastaut Syndrome. A robust clinical trial program including 3 completed phase 3 trials as well as an open-label extension study provide strong evidence to support the efficacy and safety of Epidiolex® in patients with these syndromes.

Formal clinical evaluation has shown the possibility that CBD may interact with other anti-epileptic medicines, and the precise nature of these potential interactions is being investigated through clinical trials. This means that children need to be carefully monitored for side effects to ensure their drug levels remain therapeutic and do not enter the toxic or harmful range.

In further research being conducted by GW Pharmaceuticals, other cannabinoids such as cannabidivarin (CBDV) have also been shown to promise in animal models of epilepsy and are now being investigated in clinical studies.

Other resources:

- [Medicinal Cannabis, Children's Health Queensland Hospital and Health Service](https://www.childrens.health.qld.gov.au/category/medicinal-cannabis/) (<https://www.childrens.health.qld.gov.au/category/medicinal-cannabis/>)