

Compassionate Access Scheme for Epidiolex® in children with refractory epilepsy



What is the Compassionate Access Scheme for Epidiolex®?

From July 2016, the NSW Government introduced a Compassionate Access Scheme for Epidiolex® for children with refractory epilepsy so severe that they are unable to participate in a clinical trial. Suitability for the Scheme will be as assessed by their treating Authorised Prescriber. To be eligible for the Scheme, these children must have explored all conventional treatment options and meet the agreed criteria, as assessed by their treating paediatric neurologist. The Compassionate Access Scheme for Epidiolex® is being run by the Sydney Children's Hospitals Network (SCHN) and is registered under the Therapeutics Good Administration (TGA) under the Authorised Prescriber's Scheme.

What is Epidiolex® and why is it being supplied under the Compassionate Access Scheme?

Epidiolex® is an experimental cannabis medicine which contains the non-psychoactive cannabinoid Cannabidiol (CBD). It is currently the subject of several randomised controlled trials in the United States, which are evaluating its safety and efficacy in reducing seizures in children with severe treatment-resistant epilepsy. Epidiolex® is manufactured as an oral oil-based solution and administered orally via a syringe.

Access to Epidiolex®

A doctor must first obtain permission from the TGA to become an Authorised Prescriber to prescribe Epidiolex® under the Authorised Prescriber's Scheme.

Alternative access to prescribe Epidiolex can occur through an application through the Therapeutic Goods Administration's Special Access Scheme (Category B) or may be used in a Clinical Trial. As Epidiolex® is a Schedule 4 medicine NSW Health approval is not required.

The Authorised Prescriber Scheme allows the approved doctor (Authorised Prescriber) to prescribe an unregistered cannabis medicine to a group of patients with the same symptoms or condition.

Currently, 21 NSW-based paediatric neurologists are Authorised Prescribers for Epidiolex® under the Compassionate Access Scheme. A patient who is being treated by a NSW-based paediatric neurologist may be eligible for the scheme, if referred to one of the Authorised Prescribers for Epidiolex®.

How many children are able to receive the medicine?

The Compassionate Access Scheme originally allowed for up to 40 children to be able to receive Epidiolex® at any one time. The NSW Government secured an addition 26 doses of Epidiolex® in July 2017. This means that a total of up to 66 children of the state's sickest children can be treated at any one time.

How is a child's eligibility for the Compassionate Access Scheme assessed?

Suitability for entry, or referral, into the Compassionate Access Scheme should be discussed with the child's neurologist in the first instance.

Epidiolex® and side effects

Epidiolex® appears to be relatively well tolerated by patients. However, based on information available, the most common side effects of Epidiolex® are drowsiness/sleepiness, decreased appetite, diarrhoea, increased seizures and fatigue.

Epidiolex® can interact with other epilepsy medicines. It is important that children taking Epidiolex® are routinely monitored by their usual treating paediatric neurologist to ensure that levels of other medicines are appropriately managed.

Every child is different and the ways in which they respond to specific illnesses and treatments are different. The number-one priority for the Compassionate Access Scheme is the safety of the children.

Discontinuation of treatment

Like other epilepsy medicines, data from children using Epidiolex® shows that many patients will not have a significant improvement while using Epidiolex®. Trials overseas show the condition of some patients can even deteriorate while using the medicine.

It is important that when there is little or no benefit in taking the experimental medicine, treatment is recommended to stop. There is a review at 12 weeks after first use and at each subsequent appointment to consider whether it is appropriate to continue the medicine. The children will be under the care of their treating paediatric neurologist while they trial Epidiolex® and monitored closely.

All children respond in different ways to treatment. NSW paediatric neurologists work with each family to ensure there is a plan in place for each child with severe epilepsy; this may or may not include participation in the Compassionate Access Scheme.

More information

To find out more about the Compassionate Access Scheme and clinical trials:

- Call the Help Line, **1800 217 257**
- Email the Centre for Medicinal Cannabis Research and Innovation, cmcri@moh.health.nsw.gov.au
- Write to **Cannabis Trials, Centre for Medicinal Cannabis Research and Innovation, Locked Mail Bag 961, North Sydney, NSW 2059**