



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/476469/2014
EMA/H/C/000664

EPAR summary for the public

Diacomit

stiripentol

This is a summary of the European public assessment report (EPAR) for Diacomit. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Diacomit.

What is Diacomit?

Diacomit is an anti-epileptic medicine that contains the active substance stiripentol. It is available as capsules and sachets (250 and 500 mg). The sachets contain a powder that is used to make up an oral suspension (a liquid with solid particles in it).

What is Diacomit used for?

Diacomit is used in children with a very rare type of epilepsy called 'severe myoclonic epilepsy in infancy' (SMEI), also known as Dravet's syndrome. This type of epilepsy first appears in young children during the first year of life. Diacomit is used as an add-on to clobazam and valproate (other anti-epileptic medicines) to treat generalised tonic-clonic seizures (major fits, including loss of consciousness) when these are not adequately controlled with clobazam and valproate.

Because the number of patients with SMEI is low, the disease is considered 'rare', and Diacomit was designated as an 'orphan medicine' (a medicine used in rare diseases) on 5 December 2001.

The medicine can only be obtained with a prescription.

How is Diacomit used?

Treatment with Diacomit should only be given under the supervision of a doctor who specialises in the diagnosis and management of epilepsy in children (a paediatrician or paediatric neurologist). The recommended dose is 50 mg per kilogram bodyweight, divided into two or three doses during the day.



Treatment starts with a dose of 20 mg per kg bodyweight, which is gradually increased to the recommended dose over a number of weeks depending on the patient's age. After starting Diacomit, the dose of clobazam may need to be reduced. The dose of valproate does not normally need to be adjusted.

The main studies of Diacomit were in children over three years of age. Decisions regarding the use of Diacomit in younger children should be made on a patient-by-patient basis: it can only be given to younger children if the diagnosis of SMEI has been confirmed.

Diacomit should be given at the same time as food, but not with dairy products, carbonated drinks, fruit juice, or food or drinks that contain caffeine or theophylline (a substance found in black and green tea, among other products).

How does Diacomit work?

The exact way in which stiripentol, the active substance in Diacomit, acts as an anti-epileptic medicine is not fully known. It has been shown in experimental models to increase the levels of a 'neurotransmitter' called gamma aminobutyric acid (GABA) in the brain. GABA is the main substance in nerve cells responsible for reducing the electrical activity of the brain. It is also known to amplify the effects of other anti-epileptic medicines and to slow down the rate at which they are broken down by the liver.

How has Diacomit been studied?

Diacomit has been studied in two main studies involving 65 children between three and 18 years of age. The studies compared Diacomit capsules or sachets with placebo (a dummy treatment), when they were added to the children's existing treatment with clobazam and valproate. The main measure of effectiveness was the number of patients who 'responded' to treatment. A patient was classified as a 'responder' if the number of seizures in the second month of treatment was at least 50% lower than the number in the month before treatment was started.

What benefit has Diacomit shown during the studies?

More patients responded to treatment with Diacomit than to placebo. In the first study, 71% of the patients taking Diacomit responded to treatment (15 out of 21), compared with 5% of the placebo group (1 out of 20). Similar results were seen in the second study, with 67% responding to Diacomit (8 out of 12) and 9% to placebo (1 out of 9).

What is the risk associated with Diacomit?

The most common side effects with Diacomit (seen in more than 1 in 10 patients) are loss of appetite, weight loss, insomnia (difficulty sleeping), drowsiness, ataxia (inability to co-ordinate muscle movements), hypotonia (low muscle strength) and dystonia (muscle disorders). For the full list of all side effects reported with Diacomit, see the package leaflet.

Diacomit must not be used in patients who have had psychoses (a serious mental state with a distorted sense of reality) with attacks of delirium (a mental state with confusion, excitement, restlessness and hallucinations). Care must be taken when Diacomit is used at the same time as other medicines. See the package leaflet for the full list of restrictions.

Why has Diacomit been approved?

The CHMP concluded that Diacomit had shown its effectiveness in SMEI, albeit studies were limited and did not last as long as the Committee expected. It decided that Diacomit's benefits are greater than its risks and recommended that it be given marketing authorisation.

Diacomit was originally given 'conditional approval' because there was more evidence to come about the medicine, in particular regarding its short-term and long-term effectiveness and safety. As the company has supplied the additional information necessary, the authorisation has been switched from conditional to full approval.

Other information about Diacomit

The European Commission granted a conditional marketing authorisation valid throughout the European Union for Diacomit on 4 January 2007. This was switched to a full marketing authorisation on 8 January 2014.

The full EPAR for Diacomit can be found on the Agency's website: [ema.europa.eu/Find/medicine/Human medicines/European Public Assessment Reports](http://ema.europa.eu/Find/medicine/Human%20medicines/European%20Public%20Assessment%20Reports). For more information about treatment with Diacomit, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Diacomit can be found on the Agency's website: [ema.europa.eu/Find/medicine/Human medicines/Rare disease designations](http://ema.europa.eu/Find/medicine/Human%20medicines/Rare%20disease%20designations).

This summary was last updated in 06-2014.