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Outcome of assessment on use of Epidyolex (cannabidiol)

The European Medicines Agency has finalised its assessment of an application to change the marketing authorisation for the epilepsy medicine Epidyolex. The change would have removed the requirement to use Epidyolex with another medicine called clobazam in patients with Lennox-Gastaut syndrome and Dravet syndrome.

What is Epidyolex and what is it used for?

Epidyolex is a medicine used with clobazam to treat epilepsy in patients with Lennox-Gastaut syndrome or Dravet syndrome. It is also used with other epilepsy medicines in patients with tuberous sclerosis complex (TSC).

Epidyolex has been authorised in the EU since September 2019. It contains the active substance cannabidiol and is available as a liquid to be taken by mouth.

Further information on Epidyolex's uses can be found on the Agency's website: www.ema.europa.eu/en/medicines/human/EPAR/epidyolex

What change had the company applied for?

When used in patients with Lennox-Gastaut syndrome or Dravet syndrome, Epidyolex is given in combination with clobazam. The company applied for a change so that Epidyolex could be used without clobazam.

How does Epidyolex work?

Although the way it works is not clearly understood, the active substance in Epidyolex, cannabidiol, is thought to act on targets that play a role in the movement of calcium in the cells. As this is important for the transmission of electrical signals in some nerve cells, and seizures are caused by excessive electrical activity in the brain, altering the movement of calcium is expected to reduce or prevent the seizures in patients with Lennox-Gastaut syndrome, Dravet syndrome or TSC. Cannabidiol is also thought to act on adenosine, a chemical messenger in the brain that plays an important role in suppressing seizures.

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What did the company present to support its application?

The company did not present any clinical study data in patients with Lennox-Gastaut syndrome or Dravet syndrome which had not been previously evaluated by the Agency. The company presented new analyses of some data from the four main studies which EMA's human medicines committee (CHMP) had already assessed at the time of the initial authorisation of Epidyolex. The analyses looked at outcomes in groups of patients with Lennox-Gastaut syndrome or Dravet syndrome who did not receive clobazam during the studies.

The company also submitted supportive data on the use of Epidyolex without clobazam for the treatment of TSC (which was already assessed by CHMP when Epidyolex was authorised for the treatment of TSC), and real-world data on the use of Epidyolex without clobazam for Lennox-Gastaut syndrome and Dravet syndrome collected from patients, caregivers and health insurance databases.

What were EMA's conclusions?

The Agency considered that the benefits of Epidyolex when used without clobazam have not been convincingly demonstrated. No new clinical data were submitted and the new analyses provided were not considered reliable or robust enough to show that Epidyolex will be effective on its own.

Epidyolex will therefore not be authorised for use without clobazam in the treatment of Lennox-Gastaut syndrome and Dravet syndrome.