

3 May 2017 EMA/143020/2017 Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cannabidiol for the treatment of Lennox-Gastaut syndrome

On 20 March 2017, orphan designation (EU/3/17/1855) was granted by the European Commission to GW Research Ltd, United Kingdom, for cannabidiol for the treatment of Lennox-Gastaut syndrome.

What is Lennox-Gastaut syndrome?

Lennox-Gastaut syndrome is a severe form of epilepsy that starts in childhood between 2 and 5 years of age. Patients have different types of seizures (fits), including tonic seizures (muscle contraction lasting few seconds to minutes), atypical absence seizures (during which the person has a blank stare but is still partly aware of their surroundings) and drop seizures (brief loss of muscle tone and consciousness, causing abrupt falls). Most children with Lennox-Gastaut syndrome experience some degree of learning disability and developmental delay, along with behavioural problems such as hyperactivity and aggression.

Lennox-Gastaut syndrome is long-term debilitating due to the seizures, effects on mental function and behavioural problems.

What is the estimated number of patients affected by the condition?

At the time of designation, Lennox-Gastaut syndrome affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 103,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several epilepsy medicines were authorised in the EU for treatment of seizures in children with Lennox-Gastaut syndrome, including clonazepam, felbamate, lamotrigine, rufinamide, and topiramate.

^{*}Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).



The sponsor has provided sufficient information to show that cannabidiol might be of significant benefit for patients with Lennox-Gastaut syndrome. Results from studies in patients showed that cannabidiol reduced the number of seizures in patients in whom previous treatment failed. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Cannabidiol is a substance extracted from the *Cannabis sativa* plant. Although the way this medicine works is not clearly understood, cannabidiol is thought to act on targets that play a role in the movement of calcium in the cells, which in turn is important for the transmission of electrical signals in some nerve cells. As seizures are caused by excessive electrical activity in the brain, altering the movement of calcium is expected to reduce or prevent the seizures in Lennox-Gastaut syndrome. Cannabidiol is also thought to act on adenosine, a chemical messenger in the brain that plays an important role in suppressing seizures.

What is the stage of development of this medicine?

The effects of cannabidiol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with cannabidiol in patients with Lennox-Gastaut syndrome were ongoing.

At the time of submission, cannabidiol was not authorised anywhere in the EU for Lennox-Gastaut syndrome. Orphan designation of cannabidiol had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 February 2017 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's <u>rare disease designations page</u>.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- <u>European Organisation for Rare Diseases (EURORDIS)</u>, a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cannabidiol	Treatment of Lennox-Gastaut syndrome
Bulgarian	Канабидиол	Лечение на синдром на Ленокс-Гасто
Croatian	Kanabidiol	Liječenje Lennox-Gastautova sindroma
Czech	Kanabidiol	Léčba Lennox-Gastautova syndromu
Danish	Cannabidiol	Behandling af Lennox-Gastaut syndrom
Dutch	Cannabidiol	Behandeling van het Lennox-Gastaut syndroom
Estonian	Kannabidiool	Lennox-Gastaut´ sündroomi raviks
Finnish	Kannabidioli	Lennox-Gastautin oireyhtymän hoito
French	Cannabidiol	Traitement du syndrome de Lennox-Gastaut
German	Cannabidiol	Behandlung des Lennox-Gastaut-Syndroms
Greek	Κανναβιδιόλη	θεραπεία του Συνδρόμου Lennox-Gastaut
Hungarian	Kannabidiol	Lennox-Gastaut szindróma kezelése
Italian	Cannabidiolo	Trattamento della sindrome di Lennox-Gastaut
Latvian	Kanabidiols	Lenoksa–Gasto sindroma terapija
Lithuanian	Kanabidiolis	Lenokso-Gasto sindromo gydymas
Maltese	Cannabidiol	Kura tas-sindrome ta' Lennox-Gastaut
Polish	Kannabidiol	Leczenie zespołu Lennox-Gastauta
Portuguese	Canabidiol	Tratamento do síndrome de Lennox-Gastaut
Romanian	Canabidiol	Tratamentul sindromului Lennox-Gastaut
Slovak	Kanabidiol	Liečba Lennox-Gastautovho syndrómu
Slovenian	Kanabidiol	Zdravljenje Lennox-Gastautovega sindroma
Spanish	Cannabidiol	Tratamiento del síndrome de Lennox-Gastaut
Swedish	Cannabidiol	Behandling av Lennox-Gastaut syndrom
Norwegian	Kannabidiol	Behandling av Lennox-Gastaut syndromet
Icelandic	Kannabídíól	Meðferð á Lennox-Gastaut heilkenni

¹ At the time of designation