

5 January 2018
EMA/625004/2017

Public summary of opinion on orphan designation

Cannabidiol for the treatment of West syndrome

On 16 October 2017, orphan designation (EU/3/17/1920) was granted by the European Commission to GW Research Ltd, United Kingdom, for cannabidiol for the treatment of West syndrome.

What is West syndrome?

West syndrome is an epilepsy disorder in which young children have regular seizures (fits) called 'infantile spasms'. The seizures usually start in the first year of life and can affect different parts of the body. Usually when the seizures start occurring, the child's physical and mental development suffer. Children with West syndrome also have a typical abnormal pattern of electrical activity in the brain called 'hypsarrhythmia'. While the disorder resolves in some children, the majority will have varying degrees of long-term developmental problems.

West syndrome is a long-term debilitating and life-threatening disorder because the seizures can cause severe physical and mental problems.

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

At the time of designation, West syndrome affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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, vigabatrin was authorised in the EU for the treatment of West syndrome; corticotropin (ACTH) or the related medicine tetracosactide, and corticosteroids such as prednisolone were also used. In addition, epilepsy medicines such as zonisamide and valproic acid might be given.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with West syndrome because early results showed a reduction in seizures in

*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).

patients whose condition had not improved with epilepsy medicines. 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 fully 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 West 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 the medicine have been evaluated in experimental models.

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

At the time of submission, cannabidiol was not authorised anywhere in the EU for West syndrome. Orphan designation of the medicine had been granted in the United States for treatment of infantile spasms and in both the EU and the United States for various other epilepsy syndromes such as Dravet syndrome and Lennox-Gastaut syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 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 [rare disease designations page](#).

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;
- [European Organisation for Rare Diseases \(EURORDIS\)](#), 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 West syndrome
Bulgarian	Канабидиол	Лечение на синдром на Уест
Croatian	Kanabidiol	Lijecenje Westovog sindroma
Czech	Kanabidiol	Léčba Westova syndromu
Danish	Cannabidiol	Behandling af West syndrom
Dutch	Cannabidiol	Behandeling van het syndroom van West
Estonian	Kannabidiool	Westi sündroomi ravi
Finnish	Kannabidioli	Westin oireyhtymän hoito
French	Cannabidiol	Traitement du syndrome de West
German	Cannabidiol	Behandlung des West-Syndroms
Greek	Κανναβιδιόλη	Θεραπεία του συνδρόμου West
Hungarian	Kannabidiol	West-szindróma kezelése
Italian	Cannabidiolo	Trattamento della sindrome di West
Latvian	Kanabidiols	Vesta (West) sindroma ārstēšanai
Lithuanian	Kanabidiolis	West sindromo gydymas
Maltese	Cannabidiol	Kura tas-sindrome ta' West
Polish	Kannabidiol	Leczenie zespołu Westa
Portuguese	Canabidiol	Tratamento da síndrome de West
Romanian	Canabidiol	Tratamentul sindromului West
Slovak	Kanabidiol	Liečba Westovho syndrómu
Slovenian	Kanabidiol	Zdravljenje Westovega sindroma
Spanish	Cannabidiol	Tratamiento del síndrome de West
Swedish	Cannabidiol	Behandling av Wests syndrom
Norwegian	Kannabidiol	Behandling av Wests syndrom
Icelandic	Kannabídíól	Meðferð við West-heilkenni

¹ At the time of designation