



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

10 November 2014  
EMA/COMP/557465/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Cannabidiol for the treatment of Dravet syndrome

On 15 October 2014, orphan designation (EU/3/14/1339) was granted by the European Commission to GW Pharma Ltd, United Kingdom, for cannabidiol for the treatment of Dravet syndrome.

#### What is Dravet syndrome?

Dravet syndrome, also called severe myoclonic epilepsy of infancy (SMEI), is a severe form of epilepsy that affects children and adults. It is caused by defects in genes required for the proper function of brain cells.

In Dravet syndrome, the seizures (fits) begin in the first year of life, and are most often associated with a high temperature (febrile convulsions). Later, other types of seizures typically occur, including status epilepticus (a state of continuous seizure requiring emergency medical care). From the second year of life, the child's development begins to decline or reverse, leading to problems such as impaired mental and motor (movement) skills.

Dravet syndrome is debilitating in the long term because of the poor development of mental and motor skills. It is also life threatening particularly because of the occurrence of major seizures.

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

At the time of designation, Dravet syndrome affected less than 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 26,000 people<sup>\*</sup>, 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, the medicine Diacomit (stiripentol) was authorised in the EU as add-on treatment for generalised tonic-clonic seizures (major fits, including loss of consciousness) in children with Dravet syndrome.

---

<sup>\*</sup>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 511,100,000 (Eurostat 2014).



The sponsor has provided sufficient information to show that cannabidiol might be of significant benefit for patients with Dravet syndrome because early studies show that it could reduce the number of fits in patients with this condition when used together with existing treatments. 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 a 'channel' protein called VDAC1 found on the mitochondria, the energy producing components inside cells. These channels 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 fits are caused by excessive electrical activity in the brain, altering the action of VDAC1 is expected to reduce or prevent the fits in Dravet syndrome.

### **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 Dravet syndrome were planned.

At the time of submission, cannabidiol was not authorised anywhere in the EU for Dravet syndrome. Orphan designation of the medicine 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 4 September 2014 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:

GW Pharma Ltd  
Porton Down Science Park  
Salisbury  
Wiltshire SP4 0JQ  
United Kingdom  
Tel. +44 (0)1980 557 000  
Fax +44 (0)1980 557 111  
E-mail: [medicalinformation@gwpharm.com](mailto:medicalinformation@gwpharm.com)

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cannabidiol	Treatment of Dravet syndrome
Bulgarian	канабидиол	Лечение на синдром на Dravet
Croatian	Kanabidiol	Liječenje Dravetovog sindroma
Czech	Kanabidiol	Léčba Dravetova syndromu
Danish	Cannabidiol	Behandling af Dravet syndrom
Dutch	Cannabidiol	Behandeling van het syndroom van Dravet
Estonian	Kannabidiool	Dravet' sündroomi ravi
Finnish	Kannabidioli	Dravet'n oireyhtymän hoito
French	Cannabidiol	Traitement du syndrome de Dravet
German	Cannabidiol	Behandlung des Dravet-Syndroms
Greek	Κανναβιδιόλη	Θεραπεία συνδρόμου Dravet
Hungarian	Kannabidiol	Dravet-szindróma kezelése
Italian	Cannabidiolo	Trattamento della sindrome di Dravet
Latvian	Kanabidiols	Draveta sindroma ārstēšana
Lithuanian	Kanabidiolis	Dravet sindromo gydymas
Maltese	Cannabidiol	Kura tas-sindrome ta' Dravet
Polish	Kannabidiol	Leczenie zespołu Draveta
Portuguese	Canabidiol	Tratamento da síndrome de Dravet
Romanian	Canabidiol	Tratamentul sindromului Dravet
Slovak	Kanabidiol	Liečba Dravetovej syndrómu
Slovenian	Kanabidiol	Zdravljenje Dravetovega sindroma
Spanish	Cannabidiol	Tratamiento del síndrome de Dravet
Swedish	Cannabidiol	Behandling av Dravets syndrom
Norwegian	Kannabidiol	Behandling av Dravet-syndrom
Icelandic	Kannabídíól	Meðferð við Dravet heilkenni

<sup>1</sup> At the time of designation