



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

5 January 2018  
EMA/632070/2017

## Public summary of opinion on orphan designation

### Cannabidivarin for the treatment of Rett syndrome

On 16 October 2017, orphan designation (EU/3/17/1921) was granted by the European Commission to GW Research Ltd, United Kingdom, for cannabidivarin for the treatment of Rett syndrome.

#### What is Rett syndrome?

Rett syndrome is a genetic disease characterised by intellectual disability as well as by loss of speech and regression of acquired skills between 6 and 18 months of age. Other symptoms include difficulty breathing, irregular heartbeat, a gradual loss of the ability to move, feeding difficulties, sleeping problems, constipation, repetitive hand movements and seizures (fits).

The syndrome is caused by abnormalities in the *MECP2* gene, which is important for the normal functioning of nerve cells. This gene is in the X chromosome, one of the two chromosomes (X and Y) that determine the gender. Rett syndrome is seen almost exclusively in girls (who have two X chromosomes), as the male babies (who have only one X chromosome) do not usually survive. Although the disease is genetic, most girls affected (over 95%) do not inherit it from their parents.

Rett syndrome is a seriously debilitating and life-threatening disease mainly because of problems with breathing and the heart rhythm.

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

At the time of designation, Rett syndrome affected less than 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 41,000 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, no satisfactory methods were authorised in the EU for treating Rett syndrome. Girls with the disease were given physiotherapy, speech therapy and nutritional support to

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\*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).



help relieve the symptoms of the disease. Medicines to control seizures were also used, as well as laxatives and painkillers.

### **How is this medicine expected to work?**

Although the way cannabidivarin works is not clearly understood, it is thought to help reduce inflammation and damage to glial cells (a type of brain cell that supports and protects nerve cells), to preserve the function of mitochondria (the energy producing components of cells), and to have an effect on the transmission of electrical signals in nerve cells. As seizures are caused by excessive electrical activity in the brain, this is expected to reduce the occurrence of seizures and help improve symptoms in patients with Rett syndrome.

### **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, no clinical trials with the medicine in patients with Rett syndrome had been started.

At the time of submission, cannabidivarin was not authorised anywhere in the EU for Rett 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 7 September 2017 recommending the granting of this designation.

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

Language	Active ingredient	Indication
English	Cannabidivarin	Treatment of Rett syndrome
Bulgarian	Канабидиварин	Лечение на синдром на Rett
Croatian	Kanabidivarin	Liječenje Rettovog sindroma
Czech	Kanabidivarin	Léčba Rett-syndromu
Danish	Cannabidivarin	Behandling af Rett syndrom
Dutch	Cannabidivarine	Behandeling van het Syndroom van Rett
Estonian	Kannabidivariin	Rett' sündroomi ravi
Finnish	Kannabidivarioli	Rettin oireyhtymän hoito
French	Cannabidivarine	Traitement du syndrome de Rett
German	Cannabidivarin	Behandlung des Rett-Syndroms
Greek	Κανναβιδιβαρίνη	θεραπεία του συνδρόμου Rett
Hungarian	Kannabidivarin	Rett szindróma kezelése
Italian	Cannabidivarina	Trattamento della sindrome di Rett
Latvian	Kanabidivarīns	Retta sindroma terapija
Lithuanian	Kanabidivarinąs	Rett'o sindromą gydymas
Maltese	Cannabidivarin	Kura tas-sindrome ta' Rett
Polish	Kanabidivaryna	Leczenie zespołu Retta
Portuguese	Canabidivarina	Tratamento do síndrome de Rett
Romanian	Canabidivarină	Tratamentul sindromului Rett
Slovak	Kanabidivarin	Liečba Rettovho syndrómu
Slovenian	Kanabidivarin	Zdravljenje Rettovega sindroma
Spanish	Cannabidivarina	Tratamiento del síndrome de Rett
Swedish	Cannabidivarin	Behandling av Rett syndrom
Norwegian	Kannabidivarin	Behandling av Retts syndrom
Icelandic	Kannabidívarín	Meðferð á Rett heilkenni

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