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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sarizotan hydrochloride for the treatment of Rett syndrome

On 28 July 2015, orphan designation (EU/3/15/1531) was granted by the European Commission to Newron Pharmaceuticals SpA, Italy, for sarizotan hydrochloride for the treatment of Rett syndrome.

What is Rett syndrome?

Rett syndrome is a genetic disease that is caused by abnormalities in the *MECP2* gene, which is important for the normal functioning of nerve cells. It almost exclusively affects girls. Although the disease is genetic, most girls affected (over 95%) do not inherit it from their parents.

Girls with Rett syndrome lose their ability to properly control their muscles, have feeding difficulties and learning disabilities. The features first start to appear between six and 18 months of age. Other symptoms include difficulty breathing, irregular heartbeat, sleeping problems, constipation, repetitive hand movements and seizures (fits).

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 approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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 authorised treatments were available for Rett syndrome. Girls with the disease were given physiotherapy, speech therapy and nutritional support to help relieve the symptoms of the disease. Medicines to control seizures were also used, as well as laxatives and painkillers.

[*]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 512,900,000 (Eurostat 2015).

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How is this medicine expected to work?

Girls with Rett syndrome may have low levels of serotonin, a substance in the brain and spinal cord that helps maintain a normal breathing rhythm. Sarizotan acts on certain receptors for serotonin called $5\text{-}HT_{1A}$. By stimulating these receptors, sarizotan replaces the effect of some of the missing serotonin in the brain and spinal cord. This is expected to help restore normal breathing rhythm in girls with Rett syndrome.

What is the stage of development of this medicine?

The effects of sarizotan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with sarizotan in patients with Rett syndrome had been started.

At the time of submission, sarizotan was not authorised anywhere in the EU for Rett syndrome or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 2015 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:

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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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sarizotan hydrochloride	Treatment of Rett syndrome
Bulgarian	Саризотан хидрохлорид	Лечение на синдром на Rett
Croatian	Sarizotanklorid	Liječenje Rettovog sindroma
Czech	Sarizotan hydrochloride	Léčba Rett-syndromu
Danish	Sarizotan hydrochlorid	Behandling af Rett syndrom
Dutch	Sarizotanhydrochloride	Behandeling van het Syndroom van Rett
Estonian	Sarizotan hüdrokloriid	Rett' sündroomi ravi
Finnish	Saritsotaani hydrokloridi	Rettin oireyhtymän hoito
French	Sarizotan hydrochloride	Traitement du syndrome de Rett
German	Sarizotan Hydrochlorid	Behandlung des Rett-Syndroms
Greek	Σαριζοτάνη υδροχωρική	θεραπεία του συνδρόμου Rett
Hungarian	Szarizotán hidroklorid	Rett szindróma kezelése
Italian	Sarizotan idrocloruro	Trattamento della sindrome di Rett
Latvian	Sarizontāna hidrohlorīds	Retta sindroma terapija
Lithuanian	Sarizotano hidrochloridas	Rett'o sindromo gydymas
Maltese	Sarizotan hydrochloride	Kura tas-sindrome ta' Rett
Polish	Chlorowodorek sarizotanu	Leczenie zespołu Retta
Portuguese	Cloridrato de sarizotan	Tratamento do síndrome de Rett
Romanian	Clorhidrat de sarizotan	Tratamentul sindromului Rett
Slovak	Sarizotan hydrochlorid	Liečba Rettovho syndrómu
Slovenian	Sarizotanijev hidroklorid	Zdravljenje Rettovega sindroma
Spanish	Clorhidrato de saritozan	Tratamiento del síndrome de Rett
Swedish	Sarizotan hydroklorid	Behandling av Rett syndrom
Norwegian	Sarizotan hydroklorid	Behandling av Retts syndrom
Icelandic	Sarisótan hýdróklóríð	Meðferð á Rett heilkenni

[1] At the time of designation