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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pentetrazol for the treatment of idiopathic hypersomnia

On 11 November 2015, orphan designation (EU/3/15/1569) was granted by the European Commission to Dr Jens Steinbrink, Germany, for pentetrazol for the treatment of idiopathic hypersomnia.

What is idiopathic hypersomnia?

Idiopathic hypersomnia is a sleep disorder characterised by excessive daytime sleepiness. Idiopathic means that the cause of the disease is unknown.

People with this condition struggle to stay awake during the day and feel the need to take frequent long naps. These may be prolonged or at inappropriate times, such as during a meal or a conversation, and generally don't provide any relief from the sleepiness. Most people with idiopathic hypersomnia also sleep for more than 10 hours a night and struggle to wake in the morning, because they feel very drowsy and confused upon waking.

Idiopathic hypersomnia is a long-term debilitating disease because the need for sleep during the day interferes with normal life.

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

At the time of designation, idiopathic hypersomnia affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 154,000 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 the treatment of idiopathic hypersomnia. Patients with idiopathic hypersomnia usually received advice on lifestyle changes to help regulate their sleeping pattern.

*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?

The medicine pentetrazol has been used for several years in the treatment of conditions such as respiratory disorders and low blood pressure.

Pentetrazol is a 'GABA-receptor antagonist': it blocks the action of a substance in the brain called GABA, which is thought to play a role in promoting sleep and whose function is believed to be elevated in idiopathic hypersomnia. By blocking GABA's function, the medicine is expected to reduce excessive daytime sleepiness.

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 the medicine in patients with idiopathic hypersomnia were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for idiopathic hypersomnia. Orphan designation of the medicine has been granted in the United States for idiopathic hypersomnia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 October 2015 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pentetrazol	Treatment of idiopathic hypersomnia
Bulgarian	Пентетразол	Лечение на идиопатична хиперсомния
Croatian	Pentetrazol	Liječenje idiopatske hipersomnije
Czech	Pentetrazol	Léčba idiopatické hypersomnie
Danish	Pentetrazol	Behandling af idiopatisk hypersomni
Dutch	Pentetrazol	Behandeling van idiopathische hypersomnie
Estonian	Pentetrasooli	Idiopaatilise hüpersomnia ravi
Finnish	Pentetratsoli	Idiopaattisen hypersomnian hoito
French	Pentétrazol	Traitement de l'hypersomnie idiopathique
German	Pentetrazol	Behandlung der idiopathischen Hypersomnie
Greek	Πεντετραζόλη	Θεραπεία της ιδιοπαθούς υπερυπνίας
Hungarian	Pentetrazol	Idiopathiás hypersomnia kezelése
Italian	Pentetrazolo	Trattamento dell' ipersonnia idiopatica
Latvian	Pentetrazols	Idiopātiska miegainības ārstēšana
Lithuanian	Pentetrazolas	Idiopatinės hipersomnijos gydymas
Maltese	Pentetrazol	Kura tal-ipersomnja idjopatika
Polish	Pentetrazol	Leczenie samoistnej hipersomnii
Portuguese	Pentetrazol	Tratamento de hipersonia idiopática
Romanian	Pentetrazol	Tratamentul hipersomniei idiopatice
Slovak	Pentetrazol	Liečba idiopatickej hypersomnie
Slovenian	Pentetrazol	Zdravljenje idiopatske hipersomnije
Spanish	Pentetrazol	Tratamiento de hipersomnia idiopática
Swedish	Pentetrazol	Behandling av självfakinn svefnsækni
Norwegian	Pentetrazol	Behandling av idiopatisk hypersomni
Icelandic	Pentetrazól	Meðferð sjálfvakinnar svefnsækni

[1] At the time of designation