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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mazindol for the treatment of narcolepsy

On 9 October 2015, orphan designation (EU/3/15/1547) was granted by the European Commission to NeuroLifeSciences, France, for mazindol for the treatment of narcolepsy.

What is narcolepsy?

Narcolepsy is a sleep disorder which affects the brain's ability to regulate the sleep-wake cycle. This can lead to symptoms such as irresistible urge to sleep, even at inappropriate times and places, and disturbed night-time sleep. Some patients have episodes of severe muscle weakness (cataplexy) that can cause collapse.

Narcolepsy is a long-term debilitating disease because it causes excessive daytime sleepiness and cataplexy, increasing the risk of accidents and interfering with normal life. The condition can be life-threatening.

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

At the time of designation, narcolepsy affected less than 4 in 10,000 people in the European Union (EU). This was equivalent to fewer than 205,000 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, several medicines were authorised in the EU for narcolepsy. They included modafinil and sodium oxybate. Some medicines such as methylphenidate, caffeine, clomipramine and imipramine were used off-label to treat this condition.

The sponsor has provided sufficient information to show that mazindol might be of significant benefit for patients with narcolepsy because early clinical studies showed beneficial effects in patients who did

*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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not respond to current 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?

Mazindol is an anorectic (appetite suppressant) medicine that was developed for the treatment of obesity in the 1960's. It was withdrawn, as were all anorectics, from the market in the EU at the end of the 90's but remained authorised outside the EU.

Although the way mazindol works in narcolepsy is not clearly understood, it is expected to work in a similar way to amphetamines by increasing the levels of dopamine and noradrenaline in the brain. Dopamine and noradrenaline are neurotransmitters (chemical messengers) that carry signals between brain cells, including those that promote alertness and wakefulness.

What is the stage of development of this medicine?

As mazindol is a well-known substance, the sponsor provided data from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, no clinical trials with this medicine in patients with narcolepsy had been started by the sponsor.

At the time of submission, mazindol was not authorised anywhere in the EU for narcolepsy. Orphan designation of mazindol had previously been granted in the EU for narcolepsy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 September 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

For details of the current sponsor of the orphan designation please refer to the information on the main web page of this Public Summary of Opinion.

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	Mazindol	Treatment of narcolepsy
Bulgarian	Мазиндол	Лечение на нарколепсия
Croatian	Mazindol	Liječenje narkolepsije
Czech	Mazindol	Léčba narkolepsie
Danish	Mazindol	Behandling af narkolepsi
Dutch	Mazindol	De behandeling van narcolepsie
Estonian	Masindool	Narkolepsia ravi
Finnish	Matsindoli	Narkolepsian hoito
French	Mazindole	Traitement de la narcolepsie
German	Mazindol	Behandlung von Narkolepsie
Greek	Μαζινδόλη	Θεραπεία της ναρκοληψίας
Hungarian	Mazindol	Narcolepsia kezelése
Italian	Mazindol	Trattamento della narcolessia
Latvian	Mazindols	Narkolepsijas ārstēšanai
Lithuanian	Mazindolis	Narkolepsijos gydymas
Maltese	Mazindol	Kura tan-narkolessija
Polish	Mazindol	Leczenie narkolepsji
Portuguese	Mazindol	Tratamento da narcolépsia
Romanian	Mazindolul	Tratamentul narcolepsiei
Slovak	Mazindol	Liečba narkolepsie
Slovenian	Mazindol	Zdravljenje narkolepsije
Spanish	Mazindol	Tratamiento de la narcolepsia
Swedish	Mazindol	Behandling av narkolepsi
Norwegian	Mazindol	Behandling av narkolepsi
Icelandic	Mazindól	Meðferð drómasýki

[1] At the time of designation