16 February 2010
EMA/COMP/32860/2003 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium oxybate for the treatment of narcolepsy

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in January 2010 on request of the sponsor.

On 3 February 2003, orphan designation (EU/3/02/131) was granted by the European Commission to IDIS Limited, United Kingdom, for sodium oxybate for the treatment of narcolepsy.

The sponsorship was tranferred to Celltech Pharmaceuticals Limited, United Kingdom in February 2004. Celltech Pharmaceuticals Limited subsequently changed name to UCB Pharma Limited.

What is narcolepsy?

Narcolepsy is characterised by excessive daytime sleepiness, and often disturbed night sleep with very vivid dream imagery. In addition, some of the patients experience sudden episodes of muscle weakness (called cataplexy). Narcolepsy symptoms can be very severe and damage significantly both the private and professional life of the patients. Cataplexy can increase the risk of accidents. It can occur so suddenly that the patient does not have time to prepare and avoid fall for example.

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

At the time of designation, narcolepsy affected not more than 5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of not more than 185,000 people, and is below the threshold 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?

Several products to treat the symptoms of narcolepsy were authorised in some countries in the Community at the time of submission of the application for orphan drug designation. Sodium oxybate

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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may be of potential significant benefit in patients affected by narcolepsy. This will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status

How is this medicine expected to work?

The mechanism of action of sodium oxybate (also called gamma-hydroxybutyrate) in narcolepsy is not fully understood. It may involve increased action of a substance called dopamine, which is produced in the brain, and this may improve the night sleep and indirectly ameliorate also the daytime symptoms.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials with sodium oxybate in patients with narcolepsy were completed.

The medicinal product was designated and marketed for the condition in the United Sates of America at the time of submission of the orphan designation application in the European Union.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 13 December 2002 a positive opinion recommending the grant of the above-mentioned designation.

Update: Sodium oxybate (Xyrem) has been authorised in the EU since 13 October 2005 for treatment of cataplexy in adult patients with narcolepsy.

For more information on Xyrem, see:
http://www.ema.europa.eu/humandocs/Humans/EPAR/xyrem/xyrem.htm

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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 European Union) 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

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