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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Melatonin for the treatment of non-24-hour sleep-wake disorders in blind people with no light perception

First publication	27 October 2005
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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2014 on request of the Sponsor.

On 11 April 2005, orphan designation (EU/3/05/274) was granted by the European Commission to ICON Consulting, United Kingdom, for melatonin for the treatment of non-24-hour sleep-wake disorders in blind people with no light perception.

What is non-24-hour sleep-wake disorders in blind people with no light perception?

The 24-hour rhythm in sleep and wakefulness is regulated by a "body clock". This clock is essential for the timing of various physiological rhythms. The individual cycle of this body clock is usually slightly longer than 24 hours and therefore it is reset on a daily basis to fit 24 hours. This occurs due to changes in the blood concentration of a certain substance called melatonin. Melatonin is a naturally occurring substance produced by a small flat gland localised within the brain. The production of melatonin is normally regulated by the perception of light and darkness and helps to regulate the body clock. In blind people, who cannot perceive light, this clock cannot be reset and will therefore not be synchronised with real time. These people experience alternating periods of difficulties to initiate sleep at night with difficulties to wake-up in the morning, or have early sleep onset with early awakenings.

Non-24-hour sleep-wake disorders in blind people with no light perception is a chronically debilitating condition.



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

At the time of designation, non-24-hour sleep-wake disorders in blind people with no light perception affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 93,000 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?

No satisfactory methods exist that were authorised at the time of application.

How is this medicine expected to work?

The concentration of melatonin in the blood needed to follow the daily sleep-wake rhythm will be mimicked by external administration and therefore, when given to blind individuals who cannot perceive light may adjust the daily sleep-wake rhythm and reduce the number and duration of daytime naps.

What is the stage of development of this medicine?

The effects of melatonin were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with non-24-hour sleep-wake disorders in blind people were ongoing.

The medicinal product was not marketed anywhere worldwide for non-24-hour sleep-wake disorders in blind people with no light perception, at the time of submission.

Orphan designation of melatonin was granted in the United States for treatment of circadian rhythm sleep disorders in blind people with no perception of light.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 March 2005 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.

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Melatonin	Non-24-Hour Sleep-Wake Disorder in blind people with no light perception
Czech	Melatonin	Ne 24-hodinová porucha spánku a bdění u slepých subjektů bez percepcie světla
Danish	Melatonin	Ikke-24 timers søvn-/vågenhedsforstyrrelse hos blinde uden lyssans
Dutch	Melatonine	Niet-24 uur slaap-waakstoornis in blinden zonder lichtperceptie
Estonian	Melatoniin	Mitte-24-tunnise une- ja ärkveloleku häire valgust mittetajuvatel patsientidel
Finnish	Melatoniiini	Ei 24 tunnin uni-valvehäiriö sokeilla ihmisillä, joilla ei ole ollenkaan valon havaintokykyä.
French	Mélatonine	Trouble des non-24 heures de sommeil-réveil chez le sujet aveugle n'ayant aucune perception de la lumière.
German	Melatonin	Nicht-24-Stunden-Schlaf-Wachstörungen in Blinden mit fehlender Lichtwahrnehmung
Greek	Μελατονίνη	Μη-24-ώρου διάρκειας διαταραχή του κύκλου ύπνου-εγρήγορσης σε άτομα με τύφλωση που δεν έχουν αντίληψη του φωτός
Hungarian	Melatonin	Nem 24 órás alvás-ébredés rendellenesség befolyásolása fény percepció nélküli vakság esetén
Italian	Melatonina	Disturbo del ritmo sonno-veglia diverso dalle 24 ore in soggetti totalmente ciechi.
Latvian	Melatonīns	Ne 24 stundu miega/nomoda traucējumi akliekiem cilvēkiem bez gaismas uztveres
Lithuanian	Melatoninas	Aklų asmenų be šviesos suvokimo ne 24 valandų miego ir būdravimo režimo sutrikimas
Polish	Melatonina	Niezwiązane z rytmem 24-godzinnym zaburzenia snu i czuwania u osób całkowicie niewidomych bez poczucia światła
Portuguese	Melatonina	Distúrbios de não 24 horas do ritmo sono-vigília em pessoas cegas sem percepção da luz.
Slovak	Melatonín	Porucha 24 h cyklu spánok a bdenie u slepých ľudí bez percepcie svetla
Slovenian	Melatonin	Zamik 24-urnega cikla budnosti in spanca pri slepih osebah, ki ne zaznavajo svetlobe
Spanish	Melatonina	Trastorno del ritmo sueño-vigilia no 24 horas en sujetos ciegos que no perciben la luz
Swedish	Melatonin	Icke-24 timmars sömn-/vakenhetsstörning hos blinda patienter som saknar ljusperception

¹ At the time of designation

Language	Active Ingredient	Indication
Norwegian	Melatonin	Ikke-24-timers søvn-/våkenhetsforstyrrelse hos blinde personer som ikke har lyspersepsjon
Icelandic	Melatónín	Röskun á 24 tíma mynstri svefns og vöku hjá blindum einstaklingum með enga ljósskynjun