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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

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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.	

On 23 February 2011, orphan designation (EU/3/10/841) was granted by the European Commission to Vanda Pharmaceuticals Limited, United Kingdom, for tasimelteon for the treatment of non-24-hour sleep-wake disorder in blind people with no light perception.

What is non-24-hour sleep-wake disorder?

Non-24-hour sleep-wake disorder is a condition where patients have sleep patterns that are not adjusted to the standard 24-hour clock. They fall asleep and wake up at different times compared with the general population, often in a pattern that is closer to a 25-hour clock. As a result, they have problems adjusting to the standard timetable of everyday life, often being awake or asleep at abnormal times.

The way the body adjusts to the 24-hour clock is closely linked to the pattern of daylight and night of a normal day, and people who do not perceive light, such as blind people are more likely to suffer from the disorder.

Non-24-hour sleep-wake disorder is a long-term debilitating disease because of the excessive daytime sleepiness that affects quality of life and ability to function normally in a social environment.



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

At the time of designation, non-24-hour sleep-wake disorder in blind people with no light perception affected between 1.5 and 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of between 76,000 and 112,000 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 non-24-hour sleep-wake disorder. Medicines were used to help patients sleep (such as benzodiazepine) or stay awake (caffeine) but none were specifically authorised for the condition.

How is this medicine expected to work?

A hormone called 'melatonin' plays a key role in co-ordinating the body's sleep cycle by acting on receptors in specific areas of the brain. Melatonin is produced by a gland in the brain called the pineal gland during the hours of darkness, and blood levels of the hormone are high at night and low during the day.

Tasimelteon is expected to work as a 'melatonin-receptor agonist'. This means that it attaches to the receptors that melatonin normally attaches to. By attaching itself to these receptors, it is expected to work in the same way as melatonin in promoting sleep and regulating sleep patterns.

What is the stage of development of this medicine?

The effects of tasimelteon have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with tasimelteon in the treatment of non-24-hour sleep-wake disorder in blind people with no light perception were ongoing.

At the time of submission, tasimelteon was not authorised anywhere in the EU for non-24-hour sleep-wake disorder in blind people with no light perception. Orphan designation had been granted in the United States of America for non-24 hour sleep/wake disorder in blind individuals without light perception.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2010 recommending the granting of this designation.

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Tasimeltheon	Treatment of non-24-hour sleep-wake disorders in blind people with no light perception
Bulgarian	Тазимелтеон	Синдром на смущение в не-24-часовия цикъл сън-бодрост при слепи, невъзприемащи светлина
Czech	Tasimeltheon	Ne 24-hodinová porucha spánku a bdění u slepých subjektů bez percepce světla
Danish	Tasimeltheon	Ikke-24 timers søvn-/vågenhedsforstyrrelse hos blinde uden lyssans
Dutch	Tasimeltheon	Niet-24 uur slaap-waakstoornis in blinden zonder lichtperceptie
Estonian	Tasimeltheon	Mitte-24-tunnise une- ja ärkveloleku häire valgust mittetajuvatel patsientidel
Finnish	Tasimelteoni	Ei 24 tunnin uni-valvehäiriö sokeilla ihmisillä, joilla ei ole ollenkaan valon havaintokykyä.
French	Tasimeltheon	Trouble des des desordres sommeil-veille chez le sujet aveugle n'ayant aucune perception de la lumière.
German	Tasimeltheon	Vom 24-Stundenrhythmus abweichende Schlaf-Wachstörungen bei Blinden mit fehlender Lichtwahrnehmung
Greek	Τασιμέλτεον	Μη-24-ωρου διάρκειας διαταραχή του κύκλου ύπνου-εγρήγορσης σε άτομα με τύφλωση που δεν έχουν αντίληψη του φωτός
Hungarian	Tasimeltheon	Nem 24 órás alvás-ébrenlét rendellenesség befolyásolása fény percepció nélküli vakság esetén
Italian	Tasimeltheon	Disturbo del ritmo sonno-veglia diverso dalle 24 ore in soggetti totalmente ciechi.
Latvian	Tazimelteons	24 stundu miega/nomoda traucējumu ārstēšana akliem pacientiem
Lithuanian	Tasimelteonas	Aklų asmenų be šviesos suvokimo ne 24 valandų miego ir būdravimo režimo sutrikimas
Maltese	Tasimeltheon	Kura ta' taqlib taċ-ċiklu tal-irqad u l-qawmien mhux marbutn mal-24 siegħa fil-ghomja bla hjiel ta' dawl
Polish	Tasymeltheon	Niezwiązane z rytmem 24-godzinny zaburzenia snu i czuwania u osób całkowicie niewidomych bez poczucia światła
Portuguese	Tasimeltheon	Distúrbios de não 24 horas do ritmo sono-vigília em pessoas cegas sem percepção da luz.
Romanian	Tasimeltheon	Tratamentul tulburărilor ritmului nictemeral non-24 de ore la nevăzătorii fără percepția luminii
Slovak	Tasimeltheon	Porucha 24 h cyklu spánok a bdenie u slepých ľudí bez percepcie svetla
Slovenian	Tasimeltheon	Zamik 24-urnega cikla budnosti in spanca pri slepih osebah, ki ne zaznavajo svetlobe
Spanish	Tasimeltheon	Trastorno del ritmo sueño-vigilia no 24 horas en sujetos ciegos que no perciben la luz

¹ At the time of designation

Language	Active ingredient	Indication
Swedish	Tasimelteon	Icke-24 timmars sömn-/vakenhetsstörning hos blinda patienter som saknar ljusperception
Norwegian	Tasimelteon	Ikke-24-timers søvn-/våkenhetsforstyrrelse hos blinde personer som ikke har lyspersepsjon
Icelandic	Tasimelteón	Röskun á 24 tíma mynstri svefns og vöku hjá blindum einstaklingum með enga ljósskynjun