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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Melatonin for the treatment of perinatal asphyxia

On 2 April 2012, orphan designation (EU/3/12/978) was granted by the European Commission to Dr Nicola J Robertson, United Kingdom, for melatonin for the treatment of perinatal asphyxia.

What is perinatal asphyxia?

Perinatal asphyxia happens when babies are born without enough oxygen in their blood. This is generally due to interruptions of the oxygen supplied by the mother through the placenta or the umbilical cord. Perinatal asphyxia can cause damage to the brain and other organs.

Perinatal asphyxia is a long-term debilitating disease because it can lead to the child being severely handicapped, with mental retardation and physical disabilities. It is also life threatening, as up to a fifth of the babies with the condition will die within the first days after birth.

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

At the time of designation, perinatal asphyxia affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 51,000 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 orphan designation, there was no treatment for perinatal asphyxia authorised in the EU. Babies with perinatal asphyxia received supportive treatment, and they were sometimes cooled down to a body temperature lower than normal (hypothermia) for 12 to 72 hours after birth to reduce the extent of the damage caused by the asphyxia.

[*]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. This represents a population of 506,300,000 (Eurostat 2011).

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How is this medicine expected to work?

Melatonin is a naturally occurring hormone. The exact way in which it could work in perinatal asphyxia is not fully understood, but data from experimental models show that it has a protective effect on nerve cells by increasing their survival.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of melatonin in experimental models was ongoing.

At the time of submission, no clinical trials with melatonin in patients with perinatal asphyxia had been started.

At the time of submission, melatonin was not authorised anywhere in the EU for perinatal asphyxia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 February 2012 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

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Melatonin	Treatment of perinatal asphyxia
Bulgarian	Мелатонин	Лечение на перинатална асфиксия
Czech	Melatonin	Léčba perinatální asfyxie
Danish	Melatonin	Behandling af perinatal asfyksi
Dutch	Melatonine	Behandeling van perinatale asfyxie
Estonian	Melatoniin	Perinataalne asfüksia ravi
Finnish	Melatoniini	Sikiön hapenpuutteen hoito
French	Mélatonine	Traitement de l'asphyxie périnatale
German	Melatonin	Behandlung der perinatalen Asphyxie
Greek	Μελατονίνη	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Melatonin	Perinatális asphyxia kezelése
Italian	Melatonina	Trattamento dell'asfissia perinatale
Latvian	Melatonīns	Perinatālās asfiksijas ārstēšana
Lithuanian	Melatoninas	Perinatalinės asfiksijos gydymas
Maltese	Melatonina	Kura tal-asfissija perinatali
Polish	Melatonina	Leczenie zamartwicy okołoporodowej
Portuguese	Melatonina	Tratamento da asfixia perinatal
Romanian	Melatonină	Tratamentul asfixiei perinatale
Slovak	Melatonín	Liečba perinatálnej asfyxie
Slovenian	Melatonin	Zdravljenje perinatalna asfiksija
Spanish	Melatonina	Tratamiento de la asfixia perinatal
Swedish	Melatonin	Behandling av spädbarns asfyxi
Norwegian	Melatonin	Behandling av perinatal asfyksi
Icelandic	Melatónín	Meðferð burðarmálsköfnunar

[1] At the time of designation