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Public summary of opinion on orphan designation

Melatonin for the treatment of perinatal asphyxia

On 11 January 2019, orphan designation (EU/3/18/2127) was granted by the European Commission to Therapicon S.r.l., Italy, 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 interruption in the supply of oxygen from the mother to the child through the umbilical cord. Perinatal asphyxia can cause damage to the brain and other organs.

Perinatal asphyxia is a long-term debilitating condition because it can lead to the child being mentally and physically disabled. It is also life threatening, with up to 1 baby in 5 with the condition dying within the first few days after birth.

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

At the time of designation, perinatal asphyxia affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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 were sometimes cooled down to a body temperature lower than normal (therapeutic hypothermia) for 12 to 72 hours after birth to reduce the brain damage caused by the asphyxia.

How is this medicine expected to work?

Melatonin is a natural substance produced in the body with a variety of activities. The exact way it works in perinatal asphyxia is not fully understood, but data from experimental models show that it

*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 517,400,000 (Eurostat 2018).

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can reduce the production of damaging substances in the cell following changes in oxygen levels and has a protective effect on nerve cells, increasing their survival.

What is the stage of development of this medicine?

The effects of melatonin have been evaluated in experimental models.

At the time of submission, clinical trials with melatonin in patients with perinatal asphyxia had been carried out.

At the time of submission, melatonin was not authorised anywhere in the EU for treatment of perinatal asphyxia. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2018 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

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 orphan condition 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
Croatian	Melatonin	Liječenje perinatalne asfiksije
Czech	Melatonin	Léčba perinatální asfyxie
Danish	Melatonin	Behandling af perinatal asfyksi
Dutch	Melatonine	Behandeling van perinatale asfyxie
Estonian	Melatoniin	Perinataalse 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 perinatalne asfiksije
Spanish	Melatonina	Tratamiento de la asfixia perinatal
Swedish	Melatonin	Behandling av perinatal asfyxi
Norwegian	Melatonin	Behandling av perinatal asfyksi
Icelandic	Melatónín	Meðferð burðarmálsköfnunar

[1] At the time of designation