19 May 2015
EMA/COMP/212804/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Xenon for the treatment of perinatal asphyxia

On 24 April 2015, orphan designation (EU/3/15/1483) was granted by the European Commission to Neuroprotexeon Ltd, United Kingdom, for xenon 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 mentally and physically disabled. It is also life threatening, with up to a fifth of the babies with the condition dying 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 was 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

How is this medicine expected to work?

Xenon is a type of gas found in the atmosphere in very small amounts. Xenon has been investigated as a general anaesthetic, but it is also known to have a neuroprotective effect: this means that it can help protect nerve cells from the consequences of damage that occurs, for example, when nerve cells are starved of oxygen. Xenon is thought to attach to proteins known as NMDA receptors, which are found in nerve cells. NMDA receptors have been found to be overactivated in many pathological processes, including nerve cell death. By attaching to the receptors and blocking their action, xenon is expected to provide some protection against the damage caused by perinatal asphyxia.

What is the stage of development of this medicine?

The effects of xenon have been evaluated in experimental models.

At the time of submission of the application for orphan designation, some clinical trials with xenon in patients with perinatal asphyxia had concluded and further studies were planned.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 March 2015 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:

Neuroprotexeon Ltd
52 Princes Gate
Exhibition Road
London SW7 2PG
United Kingdom
Tel. +44 (0)20 7581 4949
E-mail: info@neuroprotexeon.com

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	Xenon	Treatment of perinatal asphyxia
Bulgarian	Ксенон	Лечение на перинатална асфиксия
Croatian	Ksenon	Liječenje perinatalne asfiksije
Czech	Xenon	Léčba perinatální asfyxie
Danish	Xenon	Behandling af perinatal asfyksi
Dutch	Xenon	Behandeling van perinatale asfyxie
Estonian	Xenon	Perinataalne asfüksia ravi
Finnish	Xenon	Sikiön hapenpuutteen hoito
French	Xénon	Traitement de l'asphyxie périnatale
German	Xenon	Behandlung der perinatalen Asphyxie
Greek	Ξένο	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Xenon	Perinatális asphyxia kezelése
Italian	Xenon	Trattamento dell'asfissia perinatale
Latvian	Ksenons	Perinatālās asfiksijas ārstēšana
Lithuanian	Ksenonas	Perinatalinės asfiksijos gydymas
Maltese	Xenon	Kura tal-asfissija perinatali
Polish	Ksenon	Leczenie zamartwicy okołoporodowej
Portuguese	Xenon	Tratamento da asfixia perinatal
Romanian	Xenon	Tratamentul asfixiei perinatale
Slovak	Xenon	Liečba perinatálnej asfyxie
Slovenian	Ksenon	Zdravljenje perinatalne asfiksije
Spanish	Xenon	Tratamiento de la asfixia perinatal
Swedish	Xenon	Behandling av spädbarns asfyxi
Norwegian	Xenon	Behandling av perinatal asfyksi
Icelandic	Xenón	Meðferð burðarmálsköfnunar

[1] At the time of designation