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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-iminobiotin for the treatment of perinatal asphyxia

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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 28 January 2010, orphan designation (EU/3/09/701) was granted by the European Commission to Neurophyxia B.V., the Netherlands, for 2-iminobiotin 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 was equivalent to a total of fewer than 51,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 506,300,000 (Eurostat 2010).

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.

How is this medicine expected to work?

2-Iminobiotin is expected to work by blocking an enzyme called 'nitric oxide synthase', which is involved in the production of nitric oxide. By blocking this enzyme, 2-iminobiotin reduces the amount of nitric oxide produced.

In babies with perinatal asphyxia, too much nitric oxide is produced, especially in the 12 to 24 hours after birth, which has been shown to cause damage to the brain cells. It is expected that 2-iminobiotin given within a few hours after birth will help to reduce this damage.

What is the stage of development of this medicine?

The effects of 2-iminobiotin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the designated product in patients with perinatal asphyxia had been started.

At the time of submission, 2-iminobiotin was not authorised anywhere in the EU for perinatal asphyxia. Orphan designation of 2-iminobiotin had been granted in the United States of America for perinatal asphyxia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 November 2009 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 Community) 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 languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	2-Iminobiotin	Treatment of perinatal asphyxia
Bulgarian	2-иминобиотин	Лечение на перинатална асфиксия
Czech	2-Iminobiotin	Léčba perinatální asfyxie
Danish	2-Iminobiotin	Behandling af perinatal asfyksi
Dutch	2-Iminobiotin	Behandeling van perinatale asfyxie
Estonian	2-Iminobiotiin	Perinataalne asfüksia ravi
Finnish	2-Iminobiotiini	Sikiön hapenpuutteen hoito
French	2-Iminobiotine	Traitement de l'asphyxie périnatale
German	2-Iminobiotin	Behandlung der perinatalen Asphyxie
Greek	2-ιμινο-βιοτίνη	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	2-Iminobiotin	Perinatális asphyxia kezelése
Italian	2-Iminobiotina	Trattamento dell'asfissia perinatale
Latvian	2-Iminobiotīns	Perinatālās asfiksijas ārstēšana
Lithuanian	2-Iminobiotinas	Perinatalinės asfiksijos gydymas
Maltese	2-Iminobiotin	Kura tal-asfissija perinatali
Polish	2-Iminobiotyna	Leczenie zamartwicy okołoporodowej
Portuguese	2-Iminobiotina	Tratamento da asfixia perinatal
Romanian	2-Iminobiotin	Tratamentul asfixiei perinatale
Slovak	2-Iminobiotín	Liečba perinatálnej asfyxie
Slovenian	2-Iminobiotin	Zdravljenje perinatalna asfiksija
Spanish	2-Iminobiotina	Tratamiento de la asfixia perinatal
Swedish	2-Iminobiotin	Behandling av spädbarns asfyxi
Norwegian	2-Iminobiotin	Behandling av perinatal asfyksi
Icelandic	2-ímínóbíótín	Meðferð burðarmálsköfnunar