8 March 2017
EMA/5833/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH for the treatment of perinatal asphyxia

On 12 January 2017, orphan designation (EU/3/16/1824) was granted by the European Commission to Vect-Horus, France, for Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH (also known as VH-N439) 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 of the oxygen supplied by the mother 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 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 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 (therapeutic hypothermia) for 12 to 72 hours after birth to reduce the brain 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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

This medicine contains part of a naturally occurring substance called neurotensin attached to a compound that helps it reach the brain. In the brain it works on the part that controls body temperature, rapidly cooling the baby down.

Cooling down babies with perinatal asphyxia can reduce death and brain damage. The advantage of using a medicine, instead of methods such as cooling blankets or helmets, is that it could reduce body temperature more rapidly, help keep the temperature constant and be less stressful to the body.

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 the medicine in experimental models was ongoing.

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

At the time of submission, the medicine 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 December 2016 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 EMA website, on the medicine's rare disease designations page.

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	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Treatment of perinatal asphyxia
Bulgarian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Лечение на перинатална асфиксия
Croatian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Liječenje perinatalne asfiksije
Czech	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Léčba perinatální asfyxie
Danish	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Behandling af perinatal asfyksi
Dutch	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Behandeling van perinatale asfyxie
Estonian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Perinataalse asfüksia ravi
Finnish	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Sikiön hapenpuutteen hoito
French	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Traitement de l'asphyxie périnatale
German	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Behandlung der perinatalen Asphyxie
Greek	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Perinatális asphyxia kezelése
Italian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Trattamento dell'asfissia perinatale
Latvian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Perinatālās asfiksijas ārstēšana
Lithuanian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Perinatalinės asfiksijos gydymas
Maltese	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Kura tal-asfissija perinatali
Polish	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Leczenie zamartwicy okołoporodowej
Portuguese	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Tratamento da asfixia perinatal
Romanian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Tratamentul asfixiei perinatale
Slovak	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Liečba perinatálnej asfyxie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Zdravljenje perinatalne asfiksije
Spanish	Pr-D-Cis-Met-Pip-Arg-Leu-Arg-Sar-Cis-Lis-Arg-Pro-Tir-Tle-Leu-OH	Tratamiento de la asfixia perinatal
Swedish	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Behandling av perinatal asfyxi
Norwegian	Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH	Behandling av perinatal asfyksi
Icelandic	Pr-D-Cýs-Met-Pip-Arg-Leu-Arg-Sar-Cýs-Lýs-Arg-Pro-Týr-Tle-Leu-OH	Meðferð burðarmálsköfnunar