16 August 2018
EMA/395053/2018

Public summary of opinion on orphan designation

Argon for the treatment of perinatal asphyxia

On 27 June 2018, orphan designation (EU/3/18/2031) was granted by the European Commission to Air Liquide Santé (International), France, for argon 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 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 21,000 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.

How is this medicine expected to work?

Argon is a gas present in the atmosphere. The way argon works in babies with perinatal asphyxia is not fully understood. It is thought to reduce cell death and inflammation while activating processes

*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).

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

that repair damaged cells. In addition, laboratory studies show that argon can protect nerve cells from the damage caused by low levels of oxygen.

What is the stage of development of this medicine?

The effects of argon have been evaluated in experimental models.

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

At the time of submission, argon 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 24 May 2018 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 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	Activeingredient	Indication
English	Argon	Treatment of perinatal asphyxia
Bulgarian	Аргон	Лечение на перинатална асфиксия
Croatian	Argon	Liječenje perinatalne asfiksije
Czech	Argon	Léčba perinatální asfyxie
Danish	Argon	Behandling af perinatal asfyksi
Dutch	Argon	Behandeling van perinatale asfyxie
Estonian	Argoon	Perinataalse asfüksia ravi
Finnish	Argon	Sikiön hapenpuutteen hoito
French	Argon	Traitement de l'asphyxie périnatale
German	Argon	Behandlung der perinatalen Asphyxie
Greek	Αργό	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Argon	Perinatális asphyxia kezelése
Italian	Argon	Trattamento dell'asfissia perinatale
Latvian	Argons	Perinatālās asfiksijas ārstēšana
Lithuanian	Argonas	Perinatalinės asfiksijos gydymas
Maltese	Argon	Kura tal-asfissija perinatali
Polish	Argon	Leczenie zamartwicy okołoporodowej
Portuguese	Árgon	Tratamento da asfixia perinatal
Romanian	Argon	Tratamentul asfixiei perinatale
Slovak	Argón	Liečba perinatálnej asfyxie
Slovenian	Argon	Zdravljenje perinatalne asfiksije
Spanish	Argón	Tratamiento de la asfixia perinatal
Swedish	Argon	Behandling av perinatal asfyxi
Norwegian	Argon	Behandling av perinatal asfyksi
Icelandic	Argon	Meðferð burðarmálsköfnunar

[1] At the time of designation