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EMA/COMP/269967/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allopurinol sodium for the treatment of perinatal asphyxia

On 21 May 2015, orphan designation (EU/3/15/1493) was granted by the European Commission to ACE Pharmaceuticals BV, the Netherlands, for allopurinol sodium 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 mentally and physically disabled. 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 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 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).

How is this medicine expected to work?

Allopurinol has been used for many years for the treatment of gout. It works by blocking an enzyme called xanthine oxidase, which plays an important part in breaking down substances in the body called 'purines' into a waste product, uric acid, a process whereby certain toxic products called 'free radicals' are also formed. Although it is not well understood how the medicine works in perinatal asphyxia, it is thought to prevent the accumulation of excess free radicals in babies, which is expected to improve the symptoms of the condition.

What is the stage of development of this medicine?

The sponsor has provided non-clinical and clinical data from the published literature to support its application for orphan designation.

At the time of submission, a clinical trial with allopurinol sodium in patients with perinatal asphyxia was planned.

At the time of submission, allopurinol 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 16 April 2015 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allopurinol sodium	Treatment of perinatal asphyxia
Bulgarian	Алопуринол натрий	Лечение на перинатална асфиксия
Croatian	Alopurinolnatrij	Liječenje perinatalne asfiksije
Czech	Allopurinol sodný	Léčba perinatální asfyxie
Danish	Allopurinolnatrium	Behandling af perinatal asfyksi
Dutch	Allopurinol-natrium	Behandeling van perinatale asfyxie
Estonian	Allopurinoolnaatrium	Perinataalne asfüksia ravi
Finnish	Allopurinolinatrium	Sikiön hapenpuutteen hoito
French	Allopurinol sodique	Traitement de l'asphyxie périnatale
German	Allopurinol-Natrium	Behandlung der perinatalen Asphyxie
Greek	Αλλοπουρινόλη νατρίουχος	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Allopurinol nátrium	Perinatális asphyxia kezelése
Italian	Sodio allopurinolo	Trattamento dell'asfissia perinatale
Latvian	Allopurinola nātrija sāls	Perinatālās asfiksijas ārstēšana
Lithuanian	Alopurinolio natrio druska	Perinatalinės asfiksijos gydymas
Maltese	Allopurinol sodium	Kura tal-asfissija perinatali
Polish	Allopurynol sodu	Leczenie zamartwicy okołoporodowej
Portuguese	Sal sódico de Alopurinol	Tratamento da asfixia perinatal
Romanian	Alopurinol sare sodică	Tratamentul asfixiei perinatale
Slovak	Alopurinol sodný	Liečba perinatálnej asfyxie
Slovenian	Natrijev alopurinol	Zdravljenje perinatalna asfiksija
Spanish	Alopurinol de sodio	Tratamiento de la asfixia perinatal
Swedish	Allopurinol natrium	Behandling av spädbarns asfyxi
Norwegian	Natrijev allopurinol	Behandling av perinatal asfyksi
Icelandic	Allópúrinól natríum	Meðferð burðarmálsköfnunar

¹ At the time of designation