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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allopurinol sodium 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 6 December 2012, orphan designation (EU/3/12/1076) was granted by the European Commission to Pharmathen SA, Greece, 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 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 approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to around 36,000 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).

*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 509,000,000 (Eurostat 2012).

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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?

Allopurinol has been used for many years as tablets in the treatment of gout. It works by blocking an enzyme called xanthine oxidase, which plays an important part in converting substances in the body called purines into another substance, uric acid. The action of xanthine oxidase is thought to contribute to the damage in perinatal asphyxia. In addition, allopurinol seems to increase the amount of other substances that help protect brain cells from damage. For the treatment of perinatal asphyxia allopurinol sodium is expected to be given by injection.

What is the stage of development of this medicine?

The effects of allopurinol sodium have been evaluated in experimental models.

At the time of submission of the application for orphan designation, preliminary clinical trials with allopurinol sodium in patients with perinatal asphyxia had finished.

At the time of submission, allopurinol sodium 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 7 November 2012 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

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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[1], Norwegian and Icelandic

Language	Active substance	Indication
English	Allopurinol sodium	Treatment of perinatal asphyxia
Bulgarian	Алопуринол	Лечение на перинатална асфиксия
Czech	Allopurinol sodný	Léčba perinatální asfyxie
Danish	Allopurinol	Behandling af perinatal asfyksi
Dutch	Allopurinol-natrium	Behandeling van perinatale asfyxie
Estonian	Allopurinool	Perinataalne asfüksia ravi
Finnish	Allopurinoli	Sikiön hapenpuutteen hoito
French	Allopurinol	Traitement de l'asphyxie périnatale
German	Allopurinol	Behandlung der perinatalen Asphyxie
Greek	Αλλοπουρινόλη	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Allopurinol nátrium	Perinatális asphyxia kezelése
Italian	Sodio allopurinolo	Trattamento dell'asfissia perinatale
Latvian	Nātrija allopurinols	Perinatālās asfiksijas ārstēšana
Lithuanian	Alopurinolio natrio druska	Perinatalinės asfiksijos gydymas
Maltese	Allopurinol sodium	Kura tal-asfissija perinatali
Polish	Allopurinol sodu	Leczenie zamartwicy okołoporodowej
Portuguese	Alopurinol	Tratamento da asfixia perinatal
Romanian	Alopurinol	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	Allopurinolnatrium	Behandling av perinatal asfyksi
Icelandic	Allópúrinól natríum	Meðferð burðarmálsköfnunar

[1] At the time of designation