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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eptacog alfa (activated) for the treatment of post-neonatal intracerebral haemorrhage

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in June 2011 on request of the sponsor.

On 20 March 2007, orphan designation (EU/3/07/436) was granted by the European Commission to Novo Nordisk A/S, Denmark, for eptacog alfa (activated) for the treatment of post-neonatal intracerebral haemorrhage.

What is post-neonatal intracerebral haemorrhage?

Intracerebral haemorrhage occurs when blood spills from a small artery into the brain nervous tissue. It usually affects adults or, more rarely, children and it is most often associated with chronic hypertension and arteriosclerosis, or with malformations of the arteries in the brain. Intracerebral haemorrhage is the cause of approximately 10% of all strokes. Bleeding into the brain nervous tissue can also occur soon after birth, the so called neonatal intracerebral haemorrhage, but in this case the condition has different causes, mechanism of damage, and symptoms compared to post-neonatal intracerebral haemorrhage.

Post-neonatal intracerebral haemorrhage is a life-threatening condition, with substantial mortality in the first month; it can be chronically debilitating in survivors, many of whom are left with serious neurological problems.

What is the estimated number of patients affected by the condition?

At the time of designation, post-neonatal intracerebral haemorrhage affected approximately 3.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 142,000 people, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the

[*]Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

There were no satisfactory methods, authorised in the European Union, at the time of designation. No single surgical or medical therapy has been demonstrated to be effective in the treatment of post-neonatal intracerebral haemorrhage.

How is this medicine expected to work?

Eptacog alfa (activated) is similar in structure and function to a human coagulation factor (factor VIIa) which is normally present in the blood. Eptacog alfa (activated) is expected to boost the natural mechanism of coagulation at the site of haemorrhage, causing the formation of a stronger clot. In intracerebral haemorrhage, eptacog alfa (activated) is expected to be helpful if used immediately after the onset of symptoms, in order to prevent an enlargement of the haemorrhagic zone; this enlargement often occurs by either continuing haemorrhage, or through a subsequent (secondary) episode of haemorrhage.

What is the stage of development of this medicine?

The effects of eptacog alfa (activated) were evaluated in experimental models in animals. At the time of submission of the application for orphan designation, several clinical trials in patients with post-neonatal intracerebral haemorrhage had been initiated, and most were completed.

Eptacog alfa (activated) for inhalational use obtained Orphan Drug designation in the European Union on 14 December 2005, for the treatment of diffuse alveolar haemorrhage.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 February 2007 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

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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 Ingredient	Indication
English	Eptacog alfa (activated)	Treatment of post-neonatal intracerebral haemorrhage
Bulgarian	Ептаког алфа (активиран)	Лечение на постнеонатална интрацеребрална хеморагия
Czech	Eptacog alfa (aktivovaný)	Léčba postnatální intracerebrální hemoragie
Danish	Eptacog alfa (aktiveret)	Behandling af post-neonatal intracerebral blødning
Dutch	Eptacog alfa (geactiveerd)	Behandeling van post-neonatale intracerebrale bloeding
Estonian	Eptacog alfa (aktiviseeritud))	Postneonataalse intratserebraalse verevalumi ravi
Finnish	Eptacog alfa (aktivoitu)	Post-neonataalisen aivoverenvuodon hoito
French	Eptacog alfa (activé)	Traitement de l'hémorragie cérébrale postnatale
German	Eptacog alfa (aktiviert)	Behandlung von post-neonatalen intrazerebralen Blutungen
Greek	Eptacog alfa (ενεργοποιημένο)	Θεραπεία νεογνικής ενδοεγκεφαλικής αιμορραγίας
Hungarian	Eptacog alfa (aktivált)	Post-neonatális intracerebrális vérzés kezelése
Italian	Eptacog alfa (attivato)	Trattamento dell'emorragia intracerebrale (post-neonatale)
Latvian	Eptacog alfa (aktivēts)	Pēc neonatālas intracerebrālas asiņošanas ārstēšana
Lithuanian	Eptakogas alfa (aktyvuotas)	Postnatalinės intracerebrinės hemoragijos gydymas
Polish	Eptakog alfa (aktywowany)	Leczenie pourodzeniowego krwotoku mózgowego
Portuguese	Eptacog alfa (activado)	Tratamento da hemorragia intracerebral pós-neonatal
Romanian	Eptacog alfa (activat)	Tratamentul hemoragiei intracerebrale post-neonatale
Slovak	Eptakog alfa (aktivovaný)	Liečba postneonatálneho intracerebrálneho krvácania
Slovenian	Eptakog alfa (activirani)	Zdravljenje intracerebralne krvavitve pri novorojenčkih
Spanish	Eptacog alfa (activado)	Tratamiento de la hemorragia intracerebral post neonatal
Swedish	Eptacog alfa (aktiverad)	Behandling av postneonatal intracerebral blödning
Norwegian	Eptacog alfa (aktivert)	Behandling av post-neonatal intracerebral blødning
Icelandic	Eptacog alfa (virkjað)	Meðferð á heilablæðingu eftir nýburaskeið

[1] At the time of designation