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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eptacog alpha (activated) for the treatment of diffuse alveolar haemorrhage

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Rev.2: sponsor's change of address	29 July 2008
Rev.3: sponsor's change of address	11 August 2009
Rev.4: sponsor's change of address	16 February 2010
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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 14 December 2005, orphan designation (EU/3/05/333) was granted by the European Commission to DKA Consult ApS, Denmark, for eptacog alpha (activated) for the treatment of diffuse alveolar haemorrhage.

The sponsorship was transferred to Pharmaorigin ApS, Denmark, in May 2007.

What is diffuse alveolar haemorrhage?

Tiny air sacs called alveoli are located at the tips of the lungs. The alveoli are responsible for exchanging oxygen and carbon dioxide between air and blood, thus for breathing. Diffuse alveolar haemorrhage is a condition characterized by diffuse bleeding of the alveoli. The manifestations of alveolar hemorrhage include expectoration of blood, difficult respiration and lack of oxygenation of blood. The episodes of bleeding can be sometimes intermittent and limited. The cause of the bleeding is in most of the cases related to an underlying disease. These diseases can be autoimmune diseases, which are conditions in which an individual's defense system (immune system) starts reacting against his or her own tissues, or infections. In other cases, diffuse alveolar haemorrhage occurs following a bone marrow transplantation (a treatment consisting in replacing abnormal cells of the bone marrow with healthy cells) or as a consequence of the use of some drugs.

The bleeding in the lungs brings disturbances in the capacity of the lungs to work properly, which necessitate controlled intubations and mechanical ventilation. If the bleeding is not stopped, this will



lead to permanent reduced functioning of the lungs. Diffuse alveolar haemorrhage is a life threatening condition.

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

At the time of designation, diffuse alveolar haemorrhage affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 people*, and is below the threshold 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?

No satisfactory methods exist that were authorised at the time of the application. As initial supportive treatment, the management is focused to stop the bleeding as soon as possible and to restore the lung functioning by intubations and mechanical ventilation. Further management depends mainly on the nature of the underlying disease.

How is this medicine expected to work?

Coagulation factors are a group of proteins present in blood and needed to clot the blood. Eptacog alpha is the activated form of the coagulation factor called factor VII. It is expected that eptacog alpha (activated), delivered directly in the lungs via inhalation, might help to stop the bleeding in the lungs by initiation of the clotting mechanism.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with diffuse alveolar haemorrhage were ongoing.

Eptacog alpha (activated) was not authorised anywhere worldwide for diffuse alveolar haemorrhage or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 October 2005 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 European Union) or insufficient returns on investment.

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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:

Pharmaorigin ApS
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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	Eptacog alfa (activated)	Treatment of diffuse alveolar haemorrhage
Czech	Eptacog alfa (aktivovaný)	Léčba difusního alveolárního krvácení
Danish	Eptacog alfa (activated)	Behandling af diffus alveolær hæmorrhagi
Dutch	Eptacog alfa (geactiveerd)	Behandeling van diffuse alveolaire bloeding
Estonian	Eptacog alfa (aktiveeritud))	Diffuusse alveolaarhemorraagia ravi.
Finnish	Eptacog alfa (activoitu)	Diffuusin alveolaarisen hemorragian hoito
French	Eptacog alfa (activé)	Traitement de l' hémorragie alvéolaire diffuse
German	Eptacog alfa (aktiviert)	Behandlung der diffusen alveolaren Hämorrhagie
Greek	Eptacog alfa (ενεργοποιημένο)	Θεραπεία διάχυτης κυψελιδικής αιμορραγίας
Hungarian	Eptacog alfa (aktivált)	Diffúz alveoláris vérzés
Italian	Eptacog alfa (attivato)	Trattamento dell'emorragia alveolare diffusa
Latvian	Eptacog alfa (aktivēts)	Difūzas alveolāras asiņošanas ārstēšana
Lithuanian	Eptakog alfa (aktyvuotas)	Difuzinio alveolinio kraujavimo gydymas
Polish	Eptakog alfa (aktywowany)	Leczenie rozlanego krwawienia pęcherzykowego
Portuguese	Eptacog alfa (activated)	Tratamento de hemorragia alveolar difusa
Slovak	Eptakog alfa (aktivovaný)	Liečba difúznej alveolárnej hemorágie
Slovenian	Eptacog alfa (activated)	Zdravljenje akutne alveolarne krvavitve
Spanish	Eptacog alfa (activado)	Tratamiento de la hemorragia alveolar difusa idiopática
Swedish	Eptacog alfa (aktiverad)	Behandling av diffus alveolär blödning
Norwegian	Eptacog alfa (aktivert)	Behandling av diffus alveolær blødning
Icelandic	Eptacog alfa (virkjað)	Meðferð við dreifðri lungnablöðrublæðingu

¹ At the time of designation