



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Octocog alfa (liposomal) for the treatment of haemophilia A

On 24 July 2009, orphan designation (EU/3/09/655) was granted by the European Commission to Bayer Schering Pharma AG, Germany, for octocog alfa (liposomal) for the treatment of haemophilia A.

Bayer Schering Pharma AG changed its name to Bayer Pharma AG in October 2011.

What is haemophilia A?

Haemophilia A is an inherited bleeding disorder that is caused by the lack of a substance called factor VIII. Factor VIII is one of the human proteins involved in the blood coagulation (clotting) process. Patients with haemophilia A are more prone to bleeding than normal and have poor wound healing after injury or surgery. Bleeding can happen within muscles or the spaces in the joints, such as the elbows, knees and ankles, which can lead to permanent injury if it happens repeatedly.

Haemophilia A is a debilitating disease that is lifelong and may be life threatening because bleeding can also happen in the brain and spinal cord, the throat or the gut.

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

At the time of designation, haemophilia A affected approximately 0.6 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 30,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, medicines containing factor VIII were authorised in the EU for the treatment of haemophilia A, to replace the missing protein.

The sponsor has provided sufficient information to show that octocog alfa (liposomal) might be of significant benefit for patients with haemophilia A, because the medicine will be available as a

* 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. This represents a population of 504,800,000 (Eurostat 2009).



liposomal formulation that is expected to be given less often than currently used treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Octocog alfa (liposomal) is made of recombinant human factor VIII attached to the surface of fatty particles called 'liposomes' which act as a carrier. Octocog alfa (liposomal) is expected to work in the body in the same way as human factor VIII. By replacing the missing factor VIII, it corrects the deficiency and makes the patient less prone to bleeding.

The recombinant human factor VIII in octocog alfa (liposomal) is made by a method known as "recombinant DNA technology": it is made by a cell that has received a gene (DNA) that makes it able to produce factor VIII.

The liposome particles in this medicine have been modified by a process called 'pegylation'. This means that a chemical called polyethylene glycol has been attached to them. This is expected to decrease the rate at which the octocog alfa is cleared from the body, prolonging the protection against bleeding and allowing the medicine to be given once a week compared with three times a week for existing treatments.

What is the stage of development of this medicine?

The effects of octocog alfa (liposomal) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with haemophilia A were ongoing.

At the time of submission, octocog alfa (liposomal) was not authorised anywhere in the EU for haemophilia A or designated as 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 4 June 2009 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	Octocog alfa (liposomal)	Treatment of haemophilia A
Bulgarian	Октоког алфа (липозомален)	Лечение на хемофилия А
Czech	Octocog alfa (liposomální)	Léčba hemofilie A
Danish	Octocog alfa (liposomal)	Behandling af hæmofili A
Dutch	Octocog alfa (liposomaal)	Behandeling van hemofilie A
Estonian	Oktokoog-alfa (liposomaalne)	Hemofiilia A ravi
Finnish	Oktokogialfa (liposomaalinen)	Hemofilia A:n hoito
French	Octocog alfa (liposomal)	Traitement de l'hémophilie A
German	Octocog alfa (liposomal)	Behandlung der Hämophilie A
Greek	Octocog alfa (Λιποσωμική)	Θεραπεία της αιμορροφιλίας Α
Hungarian	Alfa-oktokog (liposzómában)	A típusú hemofília kezelése
Italian	Octocog alfa (liposomiale)	Trattamento dell'emofilia A
Latvian	Oktokogs alfa (liposomāls)	A tipa hemofilijas ārstēšana
Lithuanian	Oktokogas alfa (liposominis)	Hemofilijos A gydymas
Maltese	Octocog alfa (liposomal)	Kura ta' l-emofilja A
Polish	Oktokog alfa (liposomalny)	Leczenie hemofilii A
Portuguese	Octocog alfa (liposomal)	Tratamento da hemofilia A
Romanian	Octocog alfa (inclus în lipozomi)	Tratamentul hemofiliei A
Slovak	Oktokog alfa (lipozomálny)	Liečba hemofilie A
Slovenian	Oktokog alfa (liposomski)	Zdravljenje hemofilije A
Spanish	Octocog alfa (liposomal)	Tratamiento de la hemofilia A
Swedish	Octocog alfa (liposomal)	Behandling av hemofili A
Norwegian	Oktocog alfa (liposomal)	Behandling av hemofili A
Icelandic	Octócog alfa (í lípósómum)	Meðferð við dreyrásýki A

¹ At the time of designation