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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated recombinant factor VIII for the treatment of haemophilia A

On 26 April 2012, orphan designation (EU/3/12/995) was granted by the European Commission to Novo Nordisk A/S, Denmark, for pegylated recombinant factor VIII for the treatment of haemophilia A.

What is haemophilia A?

Haemophilia A is an inherited bleeding disorder that is caused by the lack of factor VIII, which is one of the 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 also happen within muscles or the spaces in the joints, such as the elbows, knees and ankles. This can lead to permanent injury if it happens repeatedly.

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

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

At the time of designation, haemophilia A affected approximately 0.7 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 35,000 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 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 pegylated recombinant factor VIII might be of significant benefit for patients with haemophilia A because it is expected to be given less often than current treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

[*]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 506,300,000 (Eurostat 2011).

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How is this medicine expected to work?

Pegylated recombinant factor VIII is expected to work in the body in the same way as human factor VIII. When injected into the patient's vein, it is expected to replace the missing factor VIII, thereby correcting the deficiency and making the patient less prone to bleeding.

The medicine contains factor VIII, which is made by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA) that makes the cell able to produce it. It has also been modified by a process called 'pegylation'. This means that a chemical called 'polyethylene glycol' has been attached to factor VIII. This is expected to decrease the rate at which factor VIII is removed from the body, allowing the medicine to be given less often.

What is the stage of development of this medicine?

The effects of pegylated recombinant factor VIII have been evaluated in experimental models.

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

At the time of submission, this medicine was not authorised anywhere in the EU for haemophilia A 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 8 March 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

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pegylated recombinant factor VIII	Treatment of haemophilia A
Bulgarian	Пегилиран рекомбинантен фактор VIII	Лечение на хемофилия A
Czech	Pegylovaný rekombinantní faktor VIII	Léčba hemofilie A
Danish	Pegyleret rekombinant faktor VIII	Behandling af hæmofili A
Dutch	Gepegyleerde recombinant factor VIII	Behandeling van hemofilie A
Estonian	Pegüleeritud rekombinantne faktor VIII	Hemofiilia A ravi
Finnish	Pegyloitu rekombinantti hyytymistekijä VIII	Hemofilia A:n hoito
French	Facteur VIII recombinant pegylé	Traitement de l'hémophilie A
German	Pegylierter rekombinanter Faktor VIII	Behandlung der Hämophilie A
Greek	Πεγκυλιωμένος ανασυνδυασμένος παράγοντας VIII	Θεραπεία της αιμορροφιλίας A
Hungarian	Pegilált rekombináns VIII faktor	A típusú hemofília kezelése
Italian	Fattore VIII ricombinante pegilato	Trattamento dell'emofilia A
Latvian	Pegilēts rekombinants VIII faktors	A tipa hemofīlijas ārstēšana
Lithuanian	Pegiliuotas rekombinantinis VIII faktorius	Hemofilijos A gydymas
Maltese	Fattur VIII rikombinanti peġilat	Kura ta' l-emofilja A
Polish	Pegylowany rekombinowany czynnik VIII	Leczenie hemofilii A
Portuguese	Factor VIII recombinante pegilado	Tratamento da hemofilia A
Romanian	Factor VIII recombinant pegilat	Tratamentul hemofiliei A
Slovak	Pegylovaný rekombinantný faktor VIII	Liečba hemofílie A
Slovenian	Pegiliran rekombinantni faktor VIII	Zdravljenje hemofilije A
Spanish	Factor VIII recombinante pegilado	Tratamiento de la hemofilia A
Swedish	Pegylerad rekombinant faktor VIII	Behandling av hemofili A
Norwegian	Pegylert rekombinant faktor VIII	Behandling av hemofili A
Icelandic	Pegýleraður raðbrigða storkuþáttur VIII	Meðferð við dreyrasýki A

[1] At the time of designation