European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of pegylated recombinant human factor IX for the treatment of haemophilia B

On 15 May 2009, orphan designation (EU/3/09/640) was granted by the European Commission to Novo Nordisk A/S, Denmark, for pegylated recombinant human factor IX for the treatment of haemophilia B.

What is haemophilia B?
Haemophilia B is an inherited bleeding disorder that is caused by the lack of a substance called factor IX. Factor IX is one of the human proteins involved in the blood coagulation (clotting) process. Patients with haemophilia B 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, which can lead to permanent injury if it happens repeatedly. Rare, but life-threatening bleeding can also happen in the brain and spinal cord, the throat or the gut. Haemophilia B is a lifelong debilitating disease that is life threatening.

What is the estimated number of patients affected by the condition?
At the time of designation, haemophilia B affected approximately 0.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 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 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 IX were authorised in the EU for the treatment of haemophilia B, to replace the missing protein.
The sponsor has provided sufficient information to show that pegylated recombinant human factor IX might be of significant benefit for patients with haemophilia B, because it could 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?
Recombinant human factor IX is already available in the EU as Benefix for the treatment of haemophilia B. It works in the body in the same way as human factor IX. By replacing the missing factor IX, it corrects the factor IX deficiency and gives temporary control of the bleeding disorder.

[*]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).

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Pegylated recombinant human factor IX differs from Benefix in that it has been modified by a process called 'pegylation'. This means that a chemical called polyethylene glycol has been attached to the factor IX. This is expected to decrease the rate at which the substance is removed from the body, allowing the medicine to be given less often.
Pegylated recombinant human factor IX 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 IX.

What is the stage of development of this medicine?
The effects of pegylated recombinant human factor IX have been evaluated in experimental models. At the time of submission of the application for orphan designation, no clinical trials with this medicine had been started.
At the time of submission, this medicine was not authorised anywhere in the EU for haemophilia B 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 2 April 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 Community) 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.

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Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pegylated recombinant human factor IX	Treatment of haemophilia B
Bulgarian	Пегилиран рекомбинантен човешки фактор IX	Лечение на хемофилия Б
Czech	Pegylovaný rekombinantní lidský faktor IX	Léčba hemofilie B
Danish	Pegyleret rekombinant human faktor IX	Behandling af hæmofili B
Dutch	Gepegyleerde recombinant humaan factor IX	Behandeling van hemofilie B
Estonian	Pegüleeritud rekombinantne inimese hüübimisfaktor IX	Hemofiilia B ravi
Finnish	Ihmisen pegyloitu rekombinantti hyytymistekijä IX	Hemofilia B:n hoito
French	Facteur IX humain recombinant pégylé	Traitement de l'hémophilie B
German	Pegylierter rekombinanter Human-Faktor IX	Behandlung der Hämophilie B
Greek	Pegylated ανασυνδυασμένος ανθρώπινος παράγοντας IX	Θεραπεία της αιμορροφιλίας B
Hungarian	Pegilált rekombináns emberi IX faktor	B típusú hemofília kezelése
Italian	Fattore IX umano ricombinante pegilato	Trattamento dell'emofilia B
Latvian	Pegilēts rekombinants cilvēka IX faktors	B tipa hemofilijas ārstēšana
Lithuanian	Pegiliuotas rekombinantinis žmogaus IX faktorius	Hemofilijos B gydymas
Maltese	Fattur IX uman rikombinanti pegilat	Kura ta' l-emofilja B
Polish	Pegylowany rekombinowany ludzki czynnik IX	Leczenie hemofilii B
Portuguese	Factor IX pegilado recombinante humano	Tratamento da hemofilia B
Romanian	Factor uman IX recombinant pegilat	Tratamentul hemofiliei B
Slovak	Factor IX uman recombinant pegilat	Liečba hemofílie B
Slovenian	Pegiliran rekombinantni človeški faktor IX	Zdravljenje hemofilije B
Spanish	Factor IX pegilado recombinante humano	Tratamiento de la hemofilia B
Swedish	Pegylerad rekombinant human faktor IX	Behandling av hemofili B
Norwegian	Pegylert rekombinant human faktor IX	Behandling av hemofili B
Icelandic	PEG-tengdur raðbrigða manna storkuþáttur IX	Meðferð við dreyrasýki B