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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated recombinant factor VIIa for the treatment of haemophilia B

First publication	18 August 2008
Rev.1: withdrawal from the Community Register	9 February 2015

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.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2014 on request of the Sponsor.

On 3 June 2008, orphan designation (EU/3/08/552) was granted by the European Commission to Novo Nordisk A/S, Denmark, for pegylated recombinant factor VIIa for the treatment of haemophilia B.

What is haemophilia B?

Haemophilia B is an inherited disorder where the body's ability to control blood clotting (coagulation) is impaired. It is caused by an inborn deficiency (low levels) of a substance called factor IX, which is one of the human proteins involved in the blood clotting process. Patients with haemophilia B have longer bleeding times than normal and 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 debilitating disease that is long lasting and may be life threatening.

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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 was equivalent to a total of around 5,000 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?

Several products containing factor IX are authorised for the treatment of haemophilia B in the European Union. These are used to replace the missing protein. However, not all patients with haemophilia B can benefit from these products because the immune system can react against them by producing inhibitors (antibodies) against factor IX.

In these cases, other treatments need to be used, such as medicines containing other coagulation factors, either alone or in combination. These can include factor VIIa, which works by activating another factor called factor X to start the coagulation process. Factor VIIa acts directly on factor X, independently from factor IX, so medicines containing factor VIIa can be of use in patients who have developed inhibitors to factor IX.

The sponsor has submitted satisfactory documentation to justify its assumption that pegylated recombinant factor VIIa might be of benefit for the treatment of haemophilia B, because it might contribute to the care of the haemophilia B patients who have developed inhibitors against factor IX and it may possibly be given less often than the currently used treatment. This assumption will need to be confirmed at the time of marketing authorisation to maintain the orphan status.

How is this medicine expected to work?

Pegylated recombinant factor VIIa is expected to work in the body in the same way as human factor VIIa. By activating factor X, it is expected to control the bleeding disorder in patients who have developed inhibitors to factor IX.

Pegylated recombinant factor VIIa 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 VIIa. It has also been modified by a process called 'pegylation', meaning that it has been coated with a chemical called polyethylene glycol. This decreases the rate at which the substance is removed from the body and allows the medicine to be given less often than other products that are available.

What is the stage of development of this medicine?

The effects of pegylated recombinant factor VIIa were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials were ongoing.

At the time of submission, pegylated recombinant factor VIIa was not authorised anywhere in the world for the treatment of haemophilia B or designated as orphan medicinal product elsewhere for this condition.

*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.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 April 2008 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 VIIa	Treatment of haemophilia B
Bulgarian	Пегилиран рекомбинантен фактор VIIa	Лечение на хемофилия Б
Czech	Polyethylenglykolovaný rekombinantní faktor VIIa	Léčba hemofilie B
Danish	Pegyleret rekombinant faktor VIIa	Behandling af hæmofili B
Dutch	Gepegyleerde recombinant factor VIIa	Behandeling van hemofilie B
Estonian	Pegüleeritud rekombinantne faktor VIIa	Hemofiilia B ravi
Finnish	Pegyloitu rekombinantti hyytymistekijä VIIa	Hemofilia B:n hoito
French	Facteur VIIa recombinant pegylé	Traitement de l'hémophilie B
German	Pegylierter rekombinanter Faktor VIIa	Behandlung von Hämophilie B
Greek	Pegylated ανασυνδυασμένος παράγοντας VIIa	Θεραπεία της αιμορροφιλίας B
Hungarian	Pegilált rekombináns VIIa faktor	B típusú hemofília kezelése
Italian	Fattore VIIa ricombinante pegilato	Trattamento dell'emofilia B
Latvian	Pegilēts rekombinētais VIIa faktors	B tipa hemofilijas ārstēšana
Lithuanian	Pegiliuotas rekombinantinis VIIa faktorius	Hemofilijos B gydymas
Maltese	Fattur VIIa rikombinanti peġilat	Kura ta' l-emofilja B
Polish	Pegylowany rekombinowany czynnik VIIa	Leczenie hemofilii B
Portuguese	Factor VIIa recombinante pegilado	Tratamento da hemofilia B
Romanian	Factor VIIa recombinant pegilat	Tratamentul hemofiliei B
Slovak	Pegylovaný rekombinantný faktor VIIa	Liečba hemofílie B
Slovenian	Pegiliran rekombinantni faktor VIIa	Zdravljenje hemofilije B
Spanish	Factor VIIa pegilado recombinante	Tratamiento de la hemofilia B
Swedish	Pegylerad rekombinant faktor VIIa	Behandling av hemofili B
Norwegian	Pegylert rekombinant faktor VIIa	Behandling av hemofili B
Icelandic	PEG-tengdur raðbrigðaþáttur VIIa	Meðferð við dreyrasýki B

[1] At the time of designation