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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant porcine factor VIII (B domain deleted) for the treatment of haemophilia A

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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 20 September 2010, orphan designation (EU/3/10/784) was granted by the European Commission to Inspiration Biopharmaceuticals EU Limited, Ireland, for recombinant porcine factor VIII (B domain deleted) for the treatment of haemophilia A.

The sponsorship was transferred to Baxter Innovations GmbH, Austria, in July 2013.

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 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 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 was equivalent to a total of around 30,000 people*, and is below the threshold for

*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 506,300,000 (Eurostat 2010).

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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. However, some patients with haemophilia A could not benefit from these medicines because their immune system (the body's natural defences) had reacted by producing 'inhibitors' (antibodies) against factor VIII. These inhibitors can stop the medicine from working. In these cases, other treatments were used, including treatments to try and remove the inhibitors from the blood or medicines containing other coagulation factors such as factor VIIa, which attempted to control bleeding by 'by-passing' the use of factor VIII. These methods were not effective in all patients.

The sponsor has provided sufficient information to show that recombinant porcine factor VIII (B domain deleted) might be of significant benefit for patients with haemophilia A because it might improve the treatment of patients who have developed inhibitors against human factor VIII. Early studies indicate that this medicine might be able to control bleeding episodes in these patients by restoring blood factor VIII levels. 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 porcine factor VIII (B domain deleted) is a type of factor VIII that is expected to work in the same way as human factor VIII. It is produced by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA). This makes the cell able to produce a form of factor VIII that is similar to the factor VIII normally produced by pigs.

In the body, the medicine is expected to replace the missing human factor VIII, making the patient less prone to bleeding. The medicine is therefore expected to work even in patients who have developed inhibitors against human factor VIII. This is because the pig factor VIII has a slightly different shape to human factor VIII and will not be as easily recognised by the inhibitors against the human protein.

What is the stage of development of this medicine?

The effects of recombinant porcine factor VIII (B domain deleted) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with recombinant porcine factor VIII (B domain deleted) in patients with haemophilia A were ongoing.

At the time of submission, recombinant porcine factor VIII (B domain deleted) was not authorised anywhere in the EU for haemophilia A. Orphan designation of this medicine had been granted in the United States of America for the treatment and prevention of episodic bleeding in patients with inhibitor antibodies to human coagulation factor VIII.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 June 2010 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant porcine factor VIII (B domain deleted)	Treatment of haemophilia A
Bulgarian	Рекомбинантен свински фактор VIII (премахнат В домейн)	Лечение на хемофилия А
Croatian	Rekombinantni svinjski faktor VIII (uklonjena B domena)	Liječenje hemofilije A
Czech	Rekombinantní prasečí faktor VIII (B doména vymazána)	Léčba hemofilie A
Danish	Rekombinant faktor VIII (B domæne fjernet) fra svin	Behandling af hæmofili A
Dutch	Recombinante porcine -factor VIII (zonder t B domein)	Behandeling van hemofilie A
Estonian	Rekombinantne sea faktor VIII (B domeen kustutatud)	Hemofiilia A ravi
Finnish	Rekombinantti sian hyytymistekijä VIII (B domeeni poistettu)	Hemofilia A:n hoito
French	Facteur VIII recombinant d'origine porcine (avec délètion du domaine B)	Traitement de l'hémophilie A
German	Rekombinanter porciner Faktor VIII (ohne B Domäne)	Behandlung der Hämophilie A
Greek	Ανασυνδυασμένος χοίρειος παράγοντας VIII (με απάλειψη της Β περιοχής)	Θεραπεία της αιμορροφιλίας Α
Hungarian	Rekombináns sertés faktor VIII (B domain mentes)	A típusú hemofília kezelése
Italian	Fattore VIII ricombinante porcino (privo di dominio B)	Trattamento dell'emofilia A
Latvian	Rekombinants cūku VIII koagulācijas faktors (dzēsts B domēns)	A tipa hemofīlijas ārstēšana
Lithuanian	Rekombinacinis kiaulių VIII faktorius (B domenas pašalintas)	Hemofilijos A gydymas
Maltese	Fattur VIII tal-ħnieżer rikombinanti (bil-qasam B imneħħi)	Kura ta' l-emofilja A
Polish	Rekombinowany wieprzowy czynnik VIII (pozbawiony domeny B)	Leczenie hemofilii A
Portuguese	Factor VIII Porcino Recombinante (dmínio B eliminado)	Tratamento da hemofilia A
Romanian	Factor VIII porcin recombinant (obţinut prin deleţia domeniului B)	Tratamentul hemofiliei A
Slovak	Rekombinantný prasací faktor VIII (bez B domény)	Liečba hemofílie A
Slovenian	Rekombinantni svinjski faktor VIII (brez domene B)	Zdravljenje hemofilije A

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Factor VIII porcino recombinante (con supresión del dominio B)	Tratamiento de la hemofilia A
Swedish	Rekombinant porcin factor VIII (B domän deleterad)	Behandling av hemofili A
Norwegian	Rekombinant FVIII fra svin (B domenet fjernet)	Behandling av hemofili A
Icelandic	Raðbrigða svína storkuþáttur VIII (B svæði eytt)	Meðferð við dreyrasýki A