17 November 2011
EMA/COMP/14316/2011 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated B-domain-deleted sequence-modified recombinant human factor VIII for the treatment of haemophilia A

On 23 February 2011, orphan designation (EU/3/10/847) was granted by the European Commission to Bayer Schering Pharma AG, Germany, for pegylated B-domain-deleted sequence-modified recombinant human factor VIII for the treatment of haemophilia A.

Bayer Shering 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 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 0.6 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 30,000 people, and is below the 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.

[*]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 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with haemophilia A because it 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?

Pegylated B-domain-deleted sequence-modified recombinant human 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 the factor VIII. This is expected to decrease the rate at which the substance 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 this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with haemophilia A had been started.

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 December 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:

Bayer Pharma AG
D-13342 Berlin
Germany
Telephone: +49 214 305 1348
Telefax: +49 214 305 1603
E-mail: medical-information@bayerhealtcare.com

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 B-domain-deleted sequence-modified recombinant human factor VIII	Treatment of haemophilia A
Bulgarian	Пегилиран рекомбинантен човешки фактор VIII с модифицирана секвенция с делеция на B-домена	Лечение на хемофилия A
Czech	Pegylovaný rekombinantní humánní faktor VIII s odstraněnou B-doménou a upravenou sekvencí	Léčba hemofilie A
Danish	Pegyleret B-domæne slettet sekvens modificeret rekombinant human faktor VIII	Behandling af hæmofili A
Dutch	Gepegyleerd sequentie gemodificeerd recombinant humane factor VIII met B-domein deletie	Behandeling van hemofilie A
Estonian	Pegüleeritud, järjestusest eemaldatud B-segmendiga modifitseeritud inimese rekombinantne faktor VIII	Hemofiilia A ravi
Finnish	Pegyloitu B-domeenipoistettu, sekvenssimodifioitu ihmisen rekombinantti tekijä VIII	Hemofilia A:n hoito
French	Facteur VIII humain recombinant pegylé et modifié avec deletion du domaine B	Traitement de l'hémophilie A
German	Pegylierter, sequenz-modifizierter rekombinanter humaner Faktor VIII mit Deletion der B-Domäne	Behandlung der Hämophilie A
Greek	Πεγκυλιωμένος ανασυνδυασμένος ανθρώπινος παράγοντας VIII με αφαιρεθείσα την αλληλουχία της B περιοχής	Θεραπεία της αιμορροφιλίας A
Hungarian	Pegilált törölt B-doménú módosított szekvenciájú rekombináns humán VIII. Faktor	A típusú hemofília kezelése
Italian	Fattore VIII umano ricombinante pegilato con delezione dominio B	Trattamento dell'emofilia A
Latvian	Pegilēts rekombinantais cilvēka VIII faktors ar atšķeltu B domēnu un modificētu struktūru	A tipa hemofilijas ārstēšana
Lithuanian	Pegiliuotas B-domenas - pašalinta seka - modifikuotas rekombinantinis žmogaus VIII faktorius	Hemofilijos A gydymas
Maltese	Fattur VIII uman rikombinanti peġilat bil-qasam B mħassar u b'sekwenza modifikata	Kura ta' l-emofilja A
Polish	Pegylowany, pozbawiony domeny B, ze zmodyfikowaną sekwencją, rekombinowany ludzki czynnik VIII	Leczenie hemofilii A
Portuguese	Factor VIII recombinante humano peguilado de sequência modificada com eliminação do domínio B	Tratamento da hemofilia A
Romanian	Factor de coagulare VIII recombinant uman pegilat, modificat prin deleţia domeniului B	Tratamentul hemofiliei A
Slovak	Pegylovaný rekombinantný humánny faktor VIII s odstránenou B-doménou a upravenou sekvenciou	Liečba hemofílie A
Slovenian	Pegilirani rekombinantni humani faktor VIII brez domene B in modificiranim zaporedjem	Zdravljenje hemofilije A

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Factor VIII pegilado humano recombinante modificado con supresión de secuencia del dominio B	Tratamiento de la hemofilia A
Swedish	Pegylerad, B-domän-deleterad, sekvensmodifierad rekombinant human faktor VIII	Behandling av hemofili A
Norwegian	Pegylert sekvensmodifisert rekombinant human faktor VIII der B-domenet er slettet	Behandling av hemofili A
Icelandic	Pegýleraður raðbrigða þáttur VIII úr mönnum með eyddum B-hnykli og breytta röðun	Meðferð við dreyrasýki A