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EMA/COMP/816162/2009
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant fusion protein linking human coagulation factor IX with human albumin for the treatment of haemophilia B

On 4 February 2010, orphan designation (EU/3/09/723) was granted by the European Commission to CSL Behring GmbH, Germany, for recombinant fusion protein linking human coagulation factor IX with human albumin (also known as rIX-FP) 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 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.

Haemophilia B is a debilitating disease that is lifelong and may be life threatening if bleeding happens in the brain, spinal cord, throat or gut.

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 designation, medicines containing factor IX were authorised in the EU for the treatment of haemophilia B. These medicines replace the missing protein. In some patients, other treatments were also needed, such as medicines containing other substances involved in blood clotting such as factor VIIa.

*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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The sponsor has provided sufficient information to show that recombinant fusion protein linking human coagulation factor IX with human albumin might be of significant benefit for patients with haemophilia B because the medicine 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?

The medicine is made of a copy of human factor IX, which is attached to albumin, a protein that acts as a carrier. In the body, the medicine is expected to replace the missing factor IX, making the patient less prone to bleeding. Attaching albumin to factor IX is expected to decrease the rate at which the factor IX is cleared from the body, allowing more time between injections than for medicines that contain only factor IX.

The medicine 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 linked to albumin.

What is the stage of development of this medicine?

The effects of recombinant fusion protein linking human coagulation factor IX with human albumin have been evaluated in experimental models.

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

At the time of submission, recombinant fusion protein linking human coagulation factor IX with human albumin was not authorised anywhere in the EU for haemophilia B 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 3 December 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.

For more information

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

Language	Active ingredient	Indication
English	Recombinant fusion protein linking human coagulation factor IX with human albumin	Treatment of haemophilia B
Bulgarian	Рекомбинантен фузионен протеин, свързващ човешки коагулационен фактор IX с човешки албумин	Лечение на хемофилия Б
Czech	Rekombinantní fúzní protein spojující lidský koagulačn faktor IX s lidským albuminem	Léčba hemofilie B
Danish	Rekombinant fusionsprotein som forbinder human koagulationsfaktor IX med human albumin	Behandling af hæmofili B
Dutch	Recombinant fusie-eiwit dewelke humaan stollingsfactor IX met humaan albumine verbindt	Behandeling van hemofilie B
Estonian	Rekombinantne liitvalk, mis liidab inimese IX hüübimisfaktori inimese albumiiniga	Hemofiilia B ravi
Finnish	Rekombinantti fuusioproteiini, joka koostuu ihmisen albumiiniin liitetystä ihmisen hyytymistekijästä IX	Hemofilia B:n hoito
French	Protéine de fusion recombinante liant le facteur IX de coagulation humain et l'albumine humaine	Traitement de l'hémophilie B
German	Rekombinantes Fusionsprotein aus menschlichem Gerinnungsfaktor IX und humanem Albumin	Behandlung der Hämophilie B
Greek	Ανασυνδυασμένη πρωτεΐνη σύντηξης αποτελούμενη από ανθρώπινο παράγοντα πήξης IX συνδεδεμένο με την ανθρώπινη λευκωματίνη	Θεραπεία της αιμορροφιλίας Β
Hungarian	A IX-es humán véralvadási faktort humán albuminnal összekapcsoló rekombináns fúziós fehérje.	B típusú hemofília kezelése
Italian	Proteina ricombinante di fusione, costituita da fattore IX di coagulazione del sangue umano ed albumina umana	Trattamento dell'emofilia B
Latvian	Rekombinantais konjugētais proteīns, kurš saista cilvēka IX koagulācijas faktoru saistīta ar cilvēka albumīnu	B tipa hemofīlijas ārstēšana
Lithuanian	Rekombinantinis sulietas baltymas, sujungiantis žmogaus IX krešėjimo faktorių su žmogaus albuminu	Hemofilijos B gydymas
Maltese	Proteina tal-fużjoni rikombinanti li tgħaqqad il-fattur IX tal-koagulazzjoni uman mal-albumina umana	Kura ta' l-emofilja B
Polish	Rekombinowane białko fuzyjne zawierające ludzki czynnik krzepnięcia IX połączony z ludzką albuminą	Leczenie hemofilii B
Portuguese	Proteína de fusão recombinante, ligando o factor IX de coagulação humana à albumina humana	Tratamento da hemofilia B
Romanian	Proteină recombinantă de fuziune, care leagă factorul de coagulare IX uman de albumina umană	Tratamentul hemofiliei B
Slovak	Rekombinantný fúzny proteín spájajúci ľudský koagulačný faktor IX s ľudským albumínom	Liečba hemofílie B
Slovenian	Rekombinantni fuzijski protein, sestavljen iz humanega koagulacijskega faktorja IX, povezanega s humanim albuminom	Zdravljenje hemofilije B
Spanish	Proteína recombinante de fusión que une el factor IX de coagulación humano con la albúmina humana	Tratamiento de la hemofilia B
Swedish	Rekombinant fusionsprotein som binder human koagulationsfaktor IX och humant albumin	Behandling av hemofili B
Norwegian	Rekombinant fusjonsprotein der human koagulasjonsfaktor IX er bundet til humant albumin	Behandling av hemofili B
Icelandic	Raðbrigða samrunaprótein sem samanstendur af manna storkuþætti IX sem er tengt manna albúmíni	Meðferð við dreyrasýki B