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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 for the treatment of haemophilia B (congenital factor IX deficiency)

On 8 June 2007, orphan designation (EU/3/07/453) was granted by the European Commission to Biovitrum AB, Sweden, for recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 for the treatment of haemophilia B (congenital factor IX deficiency).

The sponsorship was transferred to Biogen Idec Limited, United Kingdom, in August 2010.

What is haemophilia B (congenital factor IX deficiency)?

Haemophilia B is an inherited blood disorder where the body's ability to control blood clotting (coagulation) is impaired. Patients suffering from this condition have an in-born deficiency of coagulation factor IX, which is one of the enzymes (proteins that speed up the conversion of certain substances into other substances) that help to stabilise the blood clot by mechanically linking certain big molecules to one another and thereby increasing the strength of blood clots. In affected individuals, the blood clot is not strong enough, resulting in longer bleeding time and poor wound healing in case of injury or surgery. Bleedings may also occur into muscles or joint spaces of the elbows, knees and ankles. Recurrent bleedings in the same location may lead to permanent injury of the joint. Rare, but life-threatening bleedings may occur in the central nervous system, throat or gastro-intestinal tract. Haemophilia B is chronically debilitating and may be life threatening.

What is the estimated number of patients affected by the condition?

At the time of designation, haemophilia B affected approximately 0.2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 10,000 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).

[*]Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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What treatments are available?

Several products to treat haemophilia B (congenital factor IX deficiency) have been authorised in member states of the European Union. These products contain different levels of factor IX and aim to replace or increase the level of the deficient enzyme. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 might be of potential significant benefit for the treatment of haemophilia B, because it may provide a major contribution to patient care. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 is a synthetic construct of the coagulation factor IX and a part from IgG antibodies (proteins that are part of the immune system). When given to a patient with haemophilia B, it is expected that the product replaces the deficient enzyme and shortens and decreases the bleedings into the surrounding tissues.. It is thought that this product is more stable than pure factor IX and therefore patients could potentially be treated less frequently with this product than with currently available products.

What is the stage of development of this medicine?

The effects of recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with haemophilia B were ongoing.

Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 was not authorised anywhere worldwide for treatment of haemophilia B nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 April 2007 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 fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1	Treatment of haemophilia B (congenital factor IX deficiency)
Bulgarian	Рекомбинантен фузионен протеин, състоящ се от човешки коагулационен фактор IX, прикрепен към Fc-домена на човешки IgG1	Лечение на хемофилия B (вроден дефицит на фактор IX)
Czech	Rekombinantní fúzní protein skládající se z lidského koagulačního faktoru IX navázaného na doménu Fc lidského IgG1	Léčba hemofilie B (vrozená deficience faktoru IX)
Danish	Rekombinant fusionsprotein, som består af human koagulationsfaktor IX bundet til Fc-domænet af human IgG1	Behandling af hæmofili B (medfødt faktor IX-mangel)
Dutch	Recombinant fusie-eiwit dat bestaat uit humane stollingsfactor IX, gebonden aan het Fc-domein van humaan IgG1	Behandeling van hemofilie B (aangeboren factor-IX-deficiëntie)
Estonian	Rekombinantne liitvalk, mis koosneb inimese IX hüübimisfaktorist, mis on kokku liidetudd inimese IgG1 Fc domeeniga	B-hemofiilia (kaasasündinud IX faktori puudulikkus) ravi
Finnish	Rekombinantti fuusioproteiini, joka koostuu ihmisen IgG1:n Fc-domeeniin liitetystä ihmisen hyytymistekijästä IX	B-hemofilian hoito (hyytymistekijä IX:n perinnöllinen puute)
French	Protéine de fusion recombinante composée du facteur IX de coagulation humain lié au fragment Fc de l'IgG1	Traitement de l'hémophilie B (déficit congénital en facteur IX)
German	Rekombinantes Fusionsprotein bestehend aus menschlichem Blutgerinnungsfaktor IX gebunden an die Fc Domäne von humanem IgG1	Behandlung von Hämophilie B (angeborener Faktor-IX-Mangel)
Greek	Ανασυνδυασμένη πρωτεΐνη σύντηξης αποτελούμενη από ανθρώπινο παράγοντα πήξης IX προσαρτημένο στην περιοχή Fc της ανθρώπινης IgG1	Θεραπεία της αιμοφιλίας B (συγγενής έλλειψη παράγοντα IX)
Hungarian	Humán IgG1 Fc doménjéhez kötött IX-es humán véralvadási faktort tartalmazó rekombináns fúziós fehérje.	Haemophilia B kezelése (veleszületett IX-es faktor hiány)
Italian	Proteina di fusione ricombinante costituita dal fattore IX di coagulazione del sangue umano connesso al dominio Fc dell'IgG1 umana	Trattamento dell'emofilia B (carenza congenita del fattore IX)

[1] At the time of designation

Language	Active Ingredient	Indication
Latvian	Rekombinantais konjugētais proteīns sastāvošs no cilvēka IX koagulācijas faktora, kas pievienots cilvēka IgG1 Fc domēnam	B hemofilijas (iedzimta IX faktora deficīta) ārstēšana
Lithuanian	Rekombinantinis sulietas baltymas, susidedantis iš žmogaus IX krešėjimo faktoriaus, sulieto su žmogaus IgG1 Fc domenu	Hemofilijos B gydymas (įgimtas IX faktoriaus trūkumas)
Maltese	Proteina tal-fużjoni rikombinanti li tikkonsisti fil-fattur IX tal-koagulazzjoni uman imwaħħal mad-dominju Fc ta' l-IgG1 uman	Kura ta' l-emofilja B (defiċjenza konġenitali tal-fattur IX)
Polish	Rekombinowane białko fuzyjne składające się z ludzkiego czynnika krzepnięcia IX połączonego z domeną Fc ludzkiej IgG1	Leczenie hemofilii B (wrodzony niedobór czynnika IX)
Portuguese	Proteína recombinante de fusão que consiste no factor IX da coagulação humana ligado ao domínio Fc da IgG1 humana	Tratamento da hemofilia B (deficiência congénita de factor IX)
Romanian	Proteină recombinantă de fuziune, constituită din factorul de coagulare IX uman, care este ataşat la domeniul Fc al IgG1 umane	Tratamentul hemofiliei B (deficit congenital de factor IX)
Slovak	Rekombinantný fúzny proteín skladajúci sa z ľudského koagulačného faktora IX naviazaného na Fc fragment ľudského IgG1	Liečba hemofílie B (vrodený deficit faktora IX)
Slovenian	Rekombinantni fuzijski protein, sestavljen iz humanega koagulacijskega faktorja IX, pritrjenega na domeno Fc humanega imunoglobulina IgG1	Zdravljenje hemofilije B (kongenitalno pomanjkanje faktorja IX)
Spanish	Proteína recombinante de fusión compuesta de factor IX de coagulación humano unido al dominio Fc de la IgG1 humana	Tratamiento de la hemofilia B (deficiencia congénita del factor IX de coagulación)
Swedish	Rekombinant fusionsprotein bestående av human koagulationsfaktor IX bunden till Fc-domänen av humant IgG1	Behandling av hemofili B (medfödd faktor IX-brist)
Norwegian	Rekombinant fusjonsprotein som består av human koagulasjonsfaktor IX bundet til Fc-delen på humant IgG1	Behandling av hemofili B (medfødt faktor IX-mangel)
Icelandic	Raðbrigða samrunaprótein sem samanstendur af storkuþætti IX úr mönnum sem er bundinn Fc hluta IgG1 úr mönnum	Meðferð við dreyrasýki B (meðfæddur skortur á storkuþætti IX)