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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1 for the treatment of haemophilia A

On 20 September 2010, orphan designation (EU/3/10/783) was granted by the European Commission to Biogen Idec Limited, United Kingdom, for recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1 for the treatment of haemophilia A.

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 within 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 happen in the brain and 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 is equivalent to a total of around 30,000 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 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. Some patients needed other treatments, such as medicines containing other substances involved in blood clotting.

[*]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).

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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 the treatments that are currently being used. 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?

This medicine is made of a copy of human factor VIII, which is attached to a part of a human antibody called IgG1. In the body, the medicine is expected to replace the missing factor VIII, making the patient less prone to bleeding. The IgG1 part allows the medicine to attach to receptors on the surface of immune system cells. This allows the factor VIII to remain in the blood for longer before being cleared from the body. This is expected to allow more time between injections than for medicines that contain factor VIII on its own.

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 this fusion molecule.

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, clinical trials with this medicine in patients with haemophilia A were ongoing.

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 2 June 2010 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	Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1	Treatment of haemophilia A
Bulgarian	Рекомбинантен фузионен протеин, състоящ се от човешки коагулационен фактор VIII, прикрепен към Fc-домена на човешки IgG1	Лечение на хемофилия A
Czech	Rekombinantní fúzní protein skládající se z lidského koagulačního faktoru VIII navázaného na doménu Fc lidského IgG1	Léčba hemofilie A
Danish	Rekombinant fusionsprotein, som består af human koagulationsfaktor VIII bundet til Fc-domænet af human IgG1	Behandling af hæmofili A
Dutch	Recombinant fusie-eiwit dat bestaat uit humane stollingsfactor VIII, gebonden aan het Fc-domein van humaan IgG1	Behandeling van hemofilie A
Estonian	Rekombinantne liitvalk, mis koosneb inimese VIII hüübimisfaktorist, mis on kokku liidetudd inimese IgG1 Fc domeeniga	Hemofiilia A ravi
Finnish	Rekombinantti fuusioproteiini, joka koostuu ihmisen IgG1:n Fc-domeeniin liitetystä ihmisen hyytymistekijästä VIII	Hemofilia A:n hoito
French	Protéine de fusion recombinante composée du facteur VIII de coagulation humain lié au fragment Fc de l'IgG1	Traitement de l'hémophilie A
German	Rekombinantes Fusionsprotein bestehend aus menschlichem Blutgerinnungsfaktor VIII gebunden an die Fc Domäne von humanem IgG1	Behandlung der Hämophilie A
Greek	Ανασυνδυασμένη πρωτεΐνη σύντηξης αποτελούμενη από ανθρώπινο παράγοντα πήξης VIII προσαρτημένο στην περιοχή Fc της ανθρώπινης IgG1	Θεραπεία της αιμορροφιλίας A
Hungarian	Humán IgG1 Fc doménjéhez kötött VIII-es humán véralvadási faktort tartalmazó rekombináns fúziós fehérje.	A típusú hemofília kezelése
Italian	Proteina di fusione ricombinante costituita dal fattore VIII di coagulazione del sangue umano connesso al dominio Fc dell'IgG1 umana	Trattamento dell'emofilia A
Latvian	Rekombinantais konjugētais proteīns sastāvošs no cilvēka VIII koagulācijas faktora, kas pievienots cilvēka IgG1 Fc domēnam	A tipa hemofilijas ārstēšana
Lithuanian	Rekombinantinis sulietas baltymas, susidedantis iš žmogaus VIII krešėjimo faktoriaus, sulieto su žmogaus IgG1 Fc domenu	Hemofilijos A gydymas

[1] At the time of designation

Maltese	Proteina tal-fużjoni rikombinanti li tikkonsisti fil-fattur VIII tal-koagulazzjoni uman imwaħħal mad-dominju Fc ta' l-IgG1 uman	Kura ta' l-emofilja A
Polish	Rekombinowane białko fuzyjne składające się z ludzkiego czynnika krzepnięcia VIII połączonego z domeną Fc ludzkiej IgG1	Leczenie hemofilii A
Portuguese	Proteína recombinante de fusão que consiste no factor VIII da coagulação humana ligado ao domínio Fc da IgG1 humana	Tratamento da hemofilia A
Romanian	Proteină recombinantă de fuziune, constituită din factorul de coagulare VIII uman, care este ataşat la domeniul Fc al IgG1 umane	Tratamentul hemofiliei A
Slovak	Rekombinantný fúzny proteín skladajúci sa z ľudského koagulačného faktora VIII naviazaného na Fc fragment ľudského IgG1	Liečba hemofílie A
Slovenian	Rekombinantni fuzijski protein, sestavljen iz humanega koagulacijskega faktorja VIII, pritrjenega na domeno Fc humanega imunoglobulina IgG1	Zdravljenje hemofilije A
Spanish	Proteína recombinante de fusión compuesta de factor VIII de coagulación humano unido al dominio Fc de la IgG1 humana	Tratamiento de la hemofilia A
Swedish	Rekombinant fusionsprotein bestående av human koagulationsfaktor VIII bunden till Fc-domänen av humant IgG1	Behandling av hemofili A
Norwegian	Rekombinant fusjonsprotein som består av human koagulasjonsfaktor VIII bundet til Fc-delen på humant IgG1	Behandling av hemofili A
Icelandic	Raðbrigða samrunaprótein sem samanstendur af storkuþætti VIII úr mönnum sem er bundinn Fc hluta IgG1 úr mönnum	Meðferð við dreyrasýki A