European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF human coagulation factor X for the treatment of hereditary factor X deficiency

On 14 September 2007, orphan designation (EU/3/07/471) was granted by the European Commission to Bio Products Laboratory, United Kingdom, for human coagulation factor X for the treatment of hereditary factor X deficiency.

What is hereditary factor X deficiency?
Hereditary factor X deficiency is an inherited blood disorder (haemophilia), characterised by abnormal blood clotting that may result in abnormal bleeding. Human coagulation factor X is a protein that helps to stabilise the blood clot by mechanically linking certain big molecules to one another and thereby increasing the strength of blood clots. In patients with hereditary factor X deficiency, the blood clot is not strong enough, resulting in longer bleeding time and poor wound healing. Blood may seep into surrounding tissues, resulting in local pain and swelling. Bleeding may also occur in internal organs. Hereditary factor X deficiency is chronically debilitating and can be life threatening, since intracranial haemorrhage (bleeding in the brain) occurs in a significant proportion of affected individuals.

What are the methods of treatment available?
There are two main treatments used in the Community for the treatment of hereditary factor X deficiency: plasma (a mix of all proteins present in healthy blood) or a mix of purified coagulation proteins from plasma. Factor X is present in plasma in relatively low (and often unspecified) concentrations, so patients require quite high doses of the current treatments to make up for their specific deficiency of factor X. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product human coagulation factor X might be of potential significant benefit for the treatment of hereditary factor X deficiency, because it may possibly provide a safer option to current treatments. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

What is the estimated number of patients affected by the condition[*]?
Based on the information provided by the sponsor and previous knowledge of the Committee, hereditary factor X deficiency was considered to affect less than 0.1 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 5000 persons.

[*]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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How is this medicinal product expected to act?
Human coagulation factor X is expected to replace the deficient protein and thus it should prevent the longer bleeding times and the bleeding into the surrounding tissue in patients with hereditary factor X deficiency.

What is the stage of development of this medicinal product?
The evaluation of the effects of human coagulation factor X in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with hereditary factor X deficiency were initiated.

Human coagulation factor X was not authorised anywhere worldwide for the treatment of hereditary factor X deficiency nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 25 July 2007 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

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

Language	Active Ingredient	Indication
English	Human coagulation factor X	Treatment of hereditary factor X deficiency
Bulgarian	Човешки коагулационен фактор X	Лечение на вроден дефицит на фактор X
Czech	Lidský koagulační faktor X	Léčba pacientů s dědičným deficitem faktoru X
Danish	Human koagulationsfaktor X	Behandling af arvelig faktor X-mangel
Dutch	Humane stollingsfactor X	Behandeling van erfelijke Factor X-deficiëntie
Estonian	Inimese hüübimisfaktor X	Kaasasündinud X-faktori puudulikkuse ravi
Finnish	Ihmisen hyytymistekijä X	Hyytymistekijä X:n perinnöllisen puutoksen hoito
French	Facteur X de coagulation humain	Traitement du déficit héréditaire en facteur X
German	Humaner Gerinnungsfaktor X	Behandlung des hereditären Faktor-X-Mangels
Greek	Ανθρώπινος Παράγοντας Πήξης X	Θεραπεία της κληρονομικής ανεπάρκειας του Παράγοντα X
Hungarian	Humán véralvadási X.-faktor	Örökletes X faktor hiány kezelése
Italian	Fattore X della coagulazione, umano	Trattamento del deficit ereditario di fattore X
Latvian	Cilvēka koagulācijas faktors X	Iedzimta X faktora trūkuma ārstēšana
Lithuanian	Žmogaus X krešėjimo faktorius	Paveldėtosios X faktoriaus stokos gydymas
Maltese	Fattur X tal-koagulazzjoni uman	Kura ta' defiċjenza ereditarja tal-fattur X
Polish	Ludzki czynnik krzepnięcia X	Leczenie pacjentów z dziedzicznym niedoborem czynnika X
Portuguese	Factor X da coagulação humana	Tratamento da deficiência hereditária do Factor X
Romanian	Factor X de coagulare uman	Tratamentul deficienței ereditare de factor X
Slovak	Ľudský koagulačný faktor X	Liečba pacientov s nedostatkom koagulačného faktora X
Slovenian	Človeški koagulacijski faktor X	Zdravljenje bolnikov z dednim pomanjkanjem faktorja X
Spanish	Factor X humano de la coagulación	Tratamiento de la deficiencia hereditaria de factor X de coagulación
Swedish	Human koagulationsfaktor X	Behandling av ärftlig brist på faktor X
Norwegian	Koagulasjonsfaktor X (human)	Behandling av arvelig faktor X-mangel
Icelandic	Blóðstorkuþáttur X úr mönnum	Meðferð sjúklinga með arfgengan skort blóðstorkuþáttar X