



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

**PUBLIC SUMMARY OF
POSITIVE OPINION FOR ORPHAN DESIGNATION
OF
recombinant human factor XIII (composed of two A subunits)
for the treatment of hereditary factor XIII deficiency**

On 12 December 2003, orphan designation (EU/3/03/179) was granted by the European Commission to Chiltern International Limited, United Kingdom, for recombinant human factor XIII (composed of two A subunits) for the treatment hereditary factor XIII deficiency.

The sponsorship was transferred to Novo Nordisk A/S, Denmark, in December 2004.

What is hereditary factor XIII deficiency?

Factor XIII (FXIII) deficiency is an inherited blood disorder characterised by abnormal blood clotting that may result in abnormal bleeding. FXIII is an enzyme that helps to stabilise the blood clot by mechanically linking certain big molecules to one another and thereby increasing the mechanical strength of blood clots. In affected individuals, 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. Internal bleeding may occur. Factor XIII deficiency can be life threatening, since in a significant proportion of affected individuals bleeding in the brain tissue (intracranial haemorrhage) occurs.

What are the methods of treatment available?

Several products to treat factor XIII deficiency have been authorised in a few member states of the European Union. These products contain different levels of FXIII. Recombinant human factor XIII might be of potential significant benefit for the treatment of hereditary factor XIII deficiency, due in particular to the increased availability in all member states. The 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*?

According to the information provided by the sponsor, hereditary factor XIII deficiency was considered to affect about 190 persons in the European Union.

How is this medicinal product expected to act?

Recombinant human factor XIII is expected to replace the missing enzyme and thus it should prevent the longer bleeding times and the bleeding into the surrounding tissue in patients with hereditary factor XIII deficiency.

What is the stage of development of this medicinal product?

The effects of recombinant human factor XIII were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with hereditary factor XIII deficiency were ongoing.

Recombinant human factor XIII was not marketed anywhere worldwide for treatment of hereditary factor XIII deficiency, at the time of submission. Orphan designation of recombinant human factor XIII was granted in United States for condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 6 November 2003 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.

For more information:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Recombinant human factor XIII (composed of two A subunits)	Treatment of hereditary factor XIII deficiency
Danish	Gensplejset human XIII (dannet af to A underenheder)	Behandling af arvelig faktor XIII mangel
Dutch	Humaan recombinant-factor XIII (samengesteld uit twee A-subeenheden)	Behandeling van erfelijke factor XIII deficiëntie
Finnish	Rekombinantti ihmisen tekijä XIII (koostuu kahdesta alayksiköstä A)	Synnynnäisen tekijä XIII puutoksen hoito
French	Facteur XIII humain recombinant (composé de deux sous-unités A)	Traitement du déficit héréditaire en facteur XIII.
German	Rekombinanter humaner Blutgerinnungsfaktor XIII (bestehend aus zwei A Untereinheiten)	Behandlung von angeborenem Faktor-XIII-Mangel
Greek	Ανασυνδυασμένος ανθρώπινος παράγοντας XIII (αποτελούμενος από δύο A υπομονάδες)	Θεραπεία κληρονομικής ανεπάρκειας του παράγοντα XIII
Italian	Fattore XIII ricombinante di umano (composto da due sottounità A)	Trattamento di carenza ereditaria del fattore XIII
Portuguese	Factor XIII humano recombinante (composto por duas sub-unidades A)	Tratamento da deficiência hereditária do factor XIII
Spanish	Factor XIII humano recombinante (compuesto de dos subunidades A)	Tratamiento de la deficiencia hereditaria del factor XIII
Swedish	Rekombinant humant faktor XIII (bestående av två A-subenheter)	Behandling av ärftlig brist på faktor XIII