13 December 2013
EMA/COMP/1590/2003 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human factor XIII (composed of two A subunits) for the treatment of hereditary factor XIII deficiency

First publication	26 April 2004
Rev.1: transfer of sponsorship	12 December 2005
Rev.2: withdrawal from the Community Register	29 November 2012
Rev.3: administrative update	13 December 2013
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in July 2012 on request of the sponsor

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.

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

At the time of designation, hereditary factor XIII deficiency affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of number of around 380 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).

What treatments are 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.

How is this medicine expected to work?

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 medicine?

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.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 November 2003 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 382,800,000 (Eurostat 2003).

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 human factor XIII (composed of two A subunits)	Treatment of hereditary factor XIII deficiency
Czech	Rekombinantní humánní faktor XIII (složený ze dvou A podjednotek)	Léčba vrozeného deficitu faktoru XIII.
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
Estonian	Rekombinantne inimese faktor XIII (koosneb 2 A-alaühikust)	Kaasasündinud faktor XIII puudulikkuse ravi
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
Hungarian	Rekombináns humán XIII véralvadási factor (két A alegységet tartalmaz)	Örökletes XIII véralvadási faktor hiány kezelése
Italian	Fattore XIII ricombinante di umano (composto da due sottounità A)	Trattamento di carenza ereditaria del fattore XIII
Latvian	Rekombinēts cilvēka XIII faktors (veidots no divām A apakšvienībām)	Pārmantota XIII faktora deficīta ārstēšana
Lithuanian	Rekombinantinis XIII žmogaus faktorius (sudarytas iš dviejų A subvienetų)	Paveldimo XIII faktoriaus stokos gydymas
Polish	Rekombinowany ludzki czynnik XIII (złożony z dwóch podjednostek A)	Leczenie wrodzonego niedoboru czynnika XIII
Portuguese	Factor XIII humano recombinante (composto por duas sub-unidades A)	Tratamento da deficiência hereditária do factor XIII
Slovak	Rekombinantný humánny faktor XIII (zložený z dvoch A podjednotiek)	Liečba vrodeného deficitu faktora XIII
Slovenian	Rekombinantni humani faktor XIII (sestavljen iz dveh podenot A)	Zdravljenje dednega pomanjkanja faktorja XIII

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
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