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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human von Willebrand factor for the treatment of von Willebrand disease

On 26 November 2010, orphan designation (EU/3/10/814) was granted by the European Commission to Baxter Innovations GmbH, Austria, for recombinant human von Willebrand factor for the treatment of von Willebrand disease.

What is von Willebrand disease?

Von Willebrand disease is a hereditary bleeding disorder caused by the deficiency of von Willebrand factor, a protein in the blood that helps the blood to clot and prevents excessive bleeding.

There are various forms of the disease. Patients with very mild symptoms may be unaware of it. However, those patients with the most severe forms of the disease have excessive and prolonged bleeding that may be life threatening.

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

At the time of designation, von Willebrand disease affected less than 2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 101,000 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 designation, the main treatments authorised in the EU for von Willebrand disease were desmopressin (a synthetic hormone that stimulates the body to release more von Willebrand factor) and plasma concentrates containing von Willebrand factor.

The sponsor has provided sufficient information to show that recombinant human von Willebrand factor might be of significant benefit for patients with von Willebrand disease because early studies show that the medicine has a sustained activity over time that may lead to an improved control of bleeding

[*]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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compared with plasma-derived products. 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 expected to work by replacing the von Willebrand factor, which is missing in patients with von Willebrand disease. The replacement protein is expected to help control bleeding. The medicine is produced by a method known as 'recombinant DNA technology': it is made by cells that have received a gene (DNA), which makes them able to produce von Willebrand factor.

What is the stage of development of this medicine?

The effects of recombinant human von Willebrand factor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with recombinant human von Willebrand factor in patients with von Willebrand disease was ongoing.

At the time of submission, recombinant human von Willebrand factor was not authorised anywhere in the EU for von Willebrand disease 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 9 September 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 human von Willebrand factor	Treatment of von Willebrand disease
Bulgarian	Човешки рекомбинантен фактор на von Willebrand	Лечение на болест на von Willebrand
Czech	Lidský rekombinantní von Willebrandův faktor	Léčba von Willebrandovy choroby
Danish	Rekombinant human von Willebrand faktor	Behandling af von Willebrands sygdom
Dutch	Recombinant humane von-Willebrand factor	Behandeling van de ziekte van von Willebrand
Estonian	Inimese rekombinantne von Willebrandi faktor	Von Willebrandi tõve raviks
Finnish	Yhdistelmä-DNA-tekniikalla tuotettu ihmisen Willebrandin tekijä	Von Willebrandin taudin hoito
French	Facteur de von Willebrand recombinant humain	Traitement de la maladie de von Willebrand
German	Rekombinanter humaner Von-Willebrand-Faktor	Therapie des Von-Willebrand-Syndroms
Greek	Ανθρώπινος ανασυνδυασμένος παράγοντας von Willebrand	Θεραπεία της νόσου von Willebrand
Hungarian	Rekombináns humán von Willebrand faktor	Von Willebrand betegség kezelése
Italian	Fattore von Willebrand umano ricombinante	Trattamento della malattia di von Willebrand
Latvian	Rekombinants cilvēka Villebranda factors	Villebranda slimības ārstēšana
Lithuanian	Recombinantinis žmogaus Vilebrando faktorius	Vilebrando ligos gydymas
Maltese	Fattur ta' von Willebrand rikombinanti uman	Kura tal-marda ta' von Willebrand
Polish	Ludzki rekombinowany czynnik von Willebranda	Leczenie choroby von Willebranda
Portuguese	Factor de von Willebrand humano recombinante	Tratamento da doença de von Willebrand
Romanian	Factor von Willebrand recombinant uman	Tratamentul bolii von Willebrand
Slovak	Ľudský rekombinantný von Willebrandov faktor	Liečba von Willebrandovej choroby
Slovenian	Humani rekombinantni von Willebrandov faktor	Terapija von Willebrandove bolezni
Spanish	Factor von Willebrand humano recombinante	Tratamiento de la enfermedad de von Willebrand
Swedish	Rekombinant human von Willebrand-faktor	Behandling av von Willebrands sjukdom
Norwegian	Rekombinant human von Willebrand faktor	Behandling av von Willebrands sykdom
Icelandic	Raðbrigða manna von Willebrand storkuþáttur	Meðferð á von Willebrands sjúkdómi

[1] At the time of designation