8 November 2010
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

anti-von Willebrand aptamer for the treatment of thrombotic thrombocytopenic purpura

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in September 2010 on request of the sponsor.

On 3 June 2008, orphan designation (EU/3/08/547) was granted by the European Commission to FGK Representative Service GmbH, Germany, for anti-von Willebrand aptamer for the treatment of thrombotic thrombocytopenic purpura.

What is thrombotic thrombocytopenic purpura?

Thrombotic thrombocytopenic purpura (TTP) is a disease that is characterised by the formation of multiple blood clots in the narrow blood vessels and by low levels of platelets in the blood (thrombocytopenia). As platelets are consumed in the blood clotting process, there are fewer platelets in the blood resulting in spontaneous bleeding and bruising of the skin in purple spots (called 'purpura'). The patients develop neurological symptoms such as confusion or seizures (fits) and signs also include anaemia (low red blood cell counts) and in some cases fever.

TTP may be idiopathic, which means that the cause of the disorder is unknown. TTP may also be secondary to various other conditions such as pregnancy, infections, cancer, and drugs. A few patients have a familial form of TTP, caused by the inactivation of an enzyme called 'ADAMTS13' due to an inborn mutation (change) in the gene for the enzyme. In other patients, the immune system reacts against the enzyme and blocks its function. ADAMTS13 normally breaks down large aggregations of a substance in the body called 'von Willebrand factor', which are involved in the blood clotting process by linking to platelets. When ADAMTS13 is inactivated, the aggregations of von Willebrand factor are not broken down and more blood clots are formed in the blood vessels.

TTP is a life-threatening disease.

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

At the time of designation, thrombotic thrombocytopenic purpura affected approximately 2.2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 110,000 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?

There are no authorised medicines for TTP in the European Union (EU). At the time of submission for orphan drug designation, the standard therapy of TTP consisted of 'plasma exchange', a procedure in which the patient's blood is taken out of the body and the blood cells are separated from the liquid part (plasma). For TTP, the blood cells are returned to the patient together with plasma from another donor.

The sponsor has submitted satisfactory documentation to justify the assumption that anti-von Willebrand aptamer could be of benefit for the treatment of TTP. This assumption will need to be confirmed at the time of marketing authorisation, to maintain the orphan status.

How is this medicine expected to work?

Anti-von Willebrand aptamer consists of short pieces of modified building blocks of the genetic material (DNA and RNA) that recognise and attach to a specific part of von Willebrand factor. This prevents the factor from linking to platelets and forming blood clots, reducing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of anti-von Willebrand aptamer have been evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with TTP were ongoing.

At the time of submission, anti-von Willebrand aptamer was not authorised anywhere in the world for TTP or designated as 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 8 April 2008 recommending the granting of this designation.

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[*]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 498,000,000 (Eurostat 2006).

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	Anti-von Willebrand Aptamer	Treatment of thrombotic thrombocytopenic purpura
Bulgarian	Анти-фон Вилебранд аптамер	Лечение на Тромботична тромбоцитопенична пурпура
Czech	Anti Willebrand faktor Aptamer	Léčba trombotické trombocytopenické purpury
Danish	Anti-von Willebrand aptamer	Behandling af trombotisk trombocytopenisk purpura
Dutch	Anti- von Willebrand aptameer	Behandeling van trombotische trombocytopenische purpura
Estonian	Von Willebrandi faktori vastane aptameer	Trombootilise trombotsütopeenilise purpura ravi
Finnish	Anti- von Willebrandin tekijä-aptameeri	tromboottisen trombosytopeenisen purppuran hoito
French	Aptamère anti-Facteur Willebrand	Traitement du purpura thrombocytopénique thrombotique
German	Anti-von Willebrand Faktor Aptamer	Behandlung von thrombotisch thrombozytopenischer Purpura (Moszkowicz-Syndrom)
Greek	Απταμερές έναντι του παράγοντα von Willebrand	Θεραπεία της θρομβοτικής θρομβοκυτοπενικής πορφύρας
Hungarian	von Willebrand-faktor elleni aptamer	thromboticus thrombocytopeniás purpura kezelése
Italian	Aptamero anti-fattore von Willebrand	Trattamento della porpora trombotica trombocitopenica
Latvian	aptamērs pret fon Villebranda slimību	trombotiskās trombocitopēniskās purpura ārstēšana
Lithuanian	Aptameras prieš von Vilebrando faktorių	Trombozinės trombocitopeninės purpuros gydymas
Maltese	Anti-von Willebrand Aptamer	Kura tal-purpura trombotika trombocitopenika
Polish	Aptamer przeciw czynnikowi von Willebranda	Leczenie zakrzepowej plamicy małopłytkowej
Portuguese	Aptamer anti-von Willebrand	Tratamento da púrpura trombótica trombocitopénica .
Romanian	Aptamer anti-factor von Willebrand	Tratamentul purpurei trombotice trombocitopenice
Slovak	Aptamér proti von Willebrandovmu faktoru	Liečba trombotickej trombocytopenickej purpury
Slovenian	Proti von Willebrandov aptamer	Zdravljenje trombotične trombocitopenične purpure

[1] At the time of designation

Language	Active Ingredient	Indication
Spanish	Aptámero contra el factor de von Willebrand	Tratamiento de la púrpura trombótica trombocitopénica
Swedish	Anti-VWF-aptamer	Behandling av trombotisk trombocytopen purpura
Norwegian	Anti-von-Willebrand-aptamer	Behandling av trombotisk trombocytopenisk purpura
Icelandic	And-von Willebrand aptamer	Meðferð við blóðflagnafæðarpurpura með segamyndun