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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human soluble Fc-gamma receptor II b for the treatment of idiopathic thrombocytopenic purpura

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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.	

On 2 August 2007, orphan designation (EU/3/07/462) was granted by the European Commission to SuppreMol GmbH, Germany, for recombinant human soluble Fc-gamma receptor II b for the treatment of idiopathic thrombocytopenic purpura.

What is idiopathic thrombocytopenic purpura?

Idiopathic thrombocytopenic purpura is a disease where the affected patients' immune system reacts against certain of their own blood cells that are involved in the blood clotting process, the so-called platelets. Idiopathic means that the reason why this happens is unknown. As a result, however, there will be fewer platelets present in the blood (thrombocytopenia); resulting in spontaneous bleeding and bruising of the skin in purple spots (this symptom is called purpura). Idiopathic thrombocytopenic purpura can be acute or chronic (long lasting), and can occur in both children and adults. The severity of the bruising is determined by the degree of thrombocytopenia; it can vary from just small skin spots that occur after small injuries, to spontaneous blood losses (haemorrhages) from the nose, in the gut or in the brain (intracranial haemorrhage), which can be life threatening.

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

At the time of designation, idiopathic thrombocytopenic purpura affected between 1 and 4 in 10,000 people in the European Union (EU). This was equivalent to a total of between 50,000 and 200,000



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?

Treatment of idiopathic thrombocytopenic purpura depends on the form of the disease (acute or chronic) and on the age of onset. Current treatment methods include surgery, consisting of spleen removal (splenectomy), or pharmacological treatment (medicines). Splenectomy is performed in order to limit the destruction of the platelets, since the spleen is the most important organ where platelets are removed from the blood. Several types of medicines were authorised in the Community for the treatment of idiopathic thrombocytopenic purpura at the time of submission of the application for orphan drug designation. Recombinant human soluble Fc-gamma receptor II b could be of potential significant benefit for the treatment of idiopathic thrombocytopenic purpura because its mechanism of action. This 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?

The immune system reaction against the platelets of affected patients is mediated through protein molecules called antibodies. Antibodies are proteins that normally target and bind specific structures in the surface of foreign bodies, such as bacteria. In idiopathic thrombocytopenic purpura, there are antibodies that bind to the platelets with one end and to cells of the immune system, the cells that destroy the platelets, with the other end. Recombinant human soluble Fc-gamma receptor II b is designed to bind to the end of the antibodies that usually binds to the immune cells, thus inhibiting (blocking) the immune reaction that destroys the platelets.

What is the stage of development of this medicine?

The evaluation of the effects of recombinant human soluble Fc-gamma receptor II b in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with idiopathic thrombocytopenic purpura were initiated.

Recombinant human soluble Fc-gamma receptor II b was not authorised anywhere worldwide for the treatment idiopathic thrombocytopenic purpura or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 27 June 2007 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 (EU 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant human soluble Fc-gamma receptor IIb	Treatment of idiopathic thrombocytopenic purpura
Bulgarian	Рекомбинантен човешки разтворим Fc-gamma рецептор IIb	Лечение на идиопатична тромбоцитопенична пурпура
Czech	Rekombinantní lidský rozpustný Fc gamma receptor IIb	Léčba idiopatické trombocytopenické purpury
Danish	Rekombinant human opløselig Fc-gamma receptor IIb	Behandling af idiopatisk trombocytopenisk purpura
Dutch	Recombinant humane oplosbare Fc-gamma receptor IIb	Behandeling van ideopathische trombocytopenische purpura
Estonian	Rekombinantne humaanne lahustuv Fc-gamma-retseptor IIb	Idiopaatilise trombotsütopeenilise purpura ravi
Finnish	Rekombinanttitekniikalla tehty ihmisen liukoinen Fc-gammareseptori IIb	Idiopaattisen trombosytopeenisen purppuran hoito
French	Récepteur Fc-gamma IIb recombinant humain soluble	Traitement du purpura thrombopénique idiopathique
German	Rekombinanter humaner löslicher Fc-gamma Rezeptor IIb	Behandlung der idiopathischen thrombozytopenischen Purpura
Greek	Ανασυνδυασμένος ανθρώπινος διαλυτός Fcγ υποδοχέας IIb	Θεραπεία της Ιδιοπαθούς Θρομβοπενικής Πορφύρας.
Hungarian	Rekombináns humán oldódó IIb-típusú Fc-gamma receptor	Idiopathiás trombocytopeniás purpura kezelése
Italian	recettore Fc-gamma IIb umano solubile, ricombinante	Trattamento della Porpora Trombocitopenica Idiopatica
Latvian	Rekombinantais cilvēka šķīstošais Fc-gamma receptors IIb	Idiopātiskās trombocitopēniskās purpuras ārstēšana
Lithuanian	Tirpus rekombinantinis žmogaus Fc-gama receptorius IIb	Idiopatinės trombocitopeninės purpuros gydymas
Maltese	Riċettur Fc-gamma tat-tip IIb, rikombinanti uman solubbli	Kura tal-purpura tromboċitopenika idjopatika
Polish	Rekombinowany, ludzki, rozpuszczalny receptor Fc-gamma IIb	Leczenie idiopatycznej plamicy małopłytkowej
Portuguese	Receptor Fc gama IIb recombinante humano solúvel	Tratamento da Púrpura Trombocitopénica Idiopática
Romanian	Fc gama receptor IIb solubil uman recombinant	Tratamentul purperei trombocitopenice idiopatice
Slovak	Rekombinantný ľudský rozpustný Fc-gamma receptor IIb	Liečba idiopatickej trombocytopenickej purpury
Slovenian	Rekombinantni humani topljivi Fc-gamma receptor IIb	Zdravljenje idiopatske trombocitopenične purpуре

¹ At the time of designation

Language	Active Ingredient	Indication
Spanish	Receptor IIb Fc-gamma recombinao soluble humano	Tratamiento de la púrpura trombocitopénica idiopática
Swedish	Rekombinant human lösliq FC-gammareseptor IIb	Behandling av idiopatisk trombocytopen purpura
Norwegian	Rekombinant human løseliq Fc-gamma reseptor IIb	Behandling av idiopatisk trombocytopenisk purpura
Icelandic	Raðbrigða leysanlegur Fc-gamma viðtaki IIb úr mönnum	Til meðferðar við frumkomnum blóðflagnafæðarpurpura

Withdrawn