



## **COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

### **PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF recombinant human soluble Fc-gamma receptor II b for the treatment of idiopathic thrombocytopenic purpura**

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 are the methods of treatment 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.

#### **What is the estimated number of patients affected by the condition \* ?**

Based on the information provided by the sponsor and previous knowledge of the Committee, idiopathic thrombocytopenic purpura was considered to affect between 1 and 4 in 10,000 persons in the European Union, which, at the time of designation, corresponded to between 50,000 and 200,000 persons in total.

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\* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**How is this medicinal product expected to act?**

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 medicinal product?**

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.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 27 June 2007 a positive opinion recommending the grant of the above-mentioned designation.

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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.

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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
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 thrombocytopeniás 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 purpurei 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 purpure
Spanish	Receptor Iib Fc-gamma recombinado soluble humano	Tratamiento de la púrpura trombocitopénica idiopática
Swedish	Rekombinant human löslig FC-gammareseptor Iib	Behandling av idiopatisk trombocytopen purpura
Norwegian	Rekombinant human løselig Fc-gamma reseptor Iib	Behandling av idiopatisk trombocytopenisk purpura
Icelandic	Raðbrigða leysanlegur Fc-gamma viðtaki Iib úr mönnum	Til meðferðar við frumkomnum blóðflagnafæðarpurpura