European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 17 November 2009
Doc.Ref.: EMEA/COMP/122763/2005 Rev.1

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of recombinant megakaryopoiesis-stimulating protein for the treatment of idiopathic thrombocytopenic purpura

On 27 May 2005, orphan designation (EU/3/05/283) was granted by the European Commission to Amgen Europe BV, The Netherlands, for recombinant megakariopoiesis stimulating protein for the treatment of idiopathic thrombocytopenic purpura.

What is idiopathic thrombocytopenic purpura?
Thrombocytopenic purpura (TP) is a disease where the individual's defence system (the so-called immune system) starts to react against certain of his/her own blood cells that are involved in the blood clotting process, the so-called platelets. The TP is called "idiopathic" since the reason why the body starts reacting against its own cells is unknown. As a result, there will be less platelets present in the blood (thrombocytopenia), resulting in bruising and spontaneous bleedings (this phenomenon is called purpura). ITP can be acute or chronic and can occur in both children and adults. The severity of the bruising is determined by the degree of thrombocytopenia e.g. from tiny skin dots called petechiae that occur after small injuries to spontaneous blood losses (haemorrhages) from the nose, guts, or brain vessels that can be life threatening.

What is the estimated number of patients affected by the condition?
At the time of designation, idiopathic thrombocytopenic purpura affected approximately 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 46,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?
Treatment of ITP depends on the form of the disease (acute or chronic) and on the age of onset. Current treatment methods include surgery consisting in spleen removal (splenectomy) or medicines. Splenectomy is performed in order to limit the destruction of the platelets. Several types of medicines were authorised in the Community for the treatment of ITP at the time of submission of the application for orphan drug designation.
Recombinant megakaryopoiesis-stimulating protein could be of potential significant benefit for the treatment of idiopathic thrombocytopenic purpura because it may act in a different way than other available medicines. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

[*]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 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004).

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.europa.eu http://www.emea.europa.eu

How is this medicine expected to work?
Platelets are cells that derive from a parent cell in the bone marrow, the so-called progenitor cells which will then develop into platelets by series of cell devisions. This process is called the megakaryopoiesis. Recombinant megakaryopoiesis-stimulating protein is a medicine that might increase the number of platelets by stimulating their production from the progenitor cells.

What is the stage of development of this medicine?
The effects of recombinant megakaryopoiesis-stimulating protein were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with idiopathic thrombocytopenic purpura were ongoing.

Recombinant megakaryopoiesis-stimulating protein was not marketed anywhere worldwide for treatment of idiopathic thrombocytopenic purpura, at the time of submission. Orphan designation of Recombinant megakaryopoiesis-stimulating protein was granted in the United States for the treatment of idiopathic thrombocytopenic purpura.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 April 2005 a positive opinion recommending the grant of the above-mentioned designation.

Update: Recombinant megakariopoiesis stimulating protein (Nplate) has been authorised in the EU since 4 February 2009 for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).
Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated.
For more information on Nplate, see:
www.emea.europa.eu/humandocs/Humans/EPAR/nplate/nplate.htm

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.

For more information:
Sponsor's contact details:
Amgen Europe BV
Minervum 7061
NL 4817 ZK Breda
The Netherlands
Telephone: +31 12 23 43 61 57
Telefax: +31 12 23 42 68 14
E-mail: regulatory.compliance.elc@amgen.com

Patients' associations contact points:

DHG : Deutsche Hämophiliegesellschaft zur Bekämpfung von Blutungskrankheiten e.V.
Neumann-Reichardt-Straße 34
22041 Hamburg
Germany
Telephone: +49 40 67 22 970
Telefax: +49 40 67 24 944
E-mail: dhg@dhg.de

Morbus-Werlhof-Selbsthilfegruppe
Am Stockert 8
56316 Raubach
Germany
Telephone: +49 26 84 95 95 10
Telefax: + 49 26 84 95 94 99
E-mail: info@morbus-werlhof.de

APAI : Associazione Patologie Autoimmuni Internazionale
Via degli Uliveti 8
64029 Silvi Marina (TE)
Italy
Telephone: +39 08 59 35 35 60 / +39 33 37 02 74 20
Telefax: +39 08 59 35 35 60
E-mail: apai@assoc-apai.org

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

Language	Active Ingredient	Indication
English	Recombinant megakaryopoeisis-stimulating protein	Treatment of idiopathic thrombocytopenic purpura
Czech	Rekombinantní megakaryopoezu stimulující protein	Léčba idiopatické trombocytopenické purpury
Danish	Rekombinant megacaryopoese stimulerende protein	Behandling af idiopatisk trombocytopenisk purpura
Dutch	Recombinant megakaryopoëse-stimulerend proteïne	Behandeling van ideopathische thrombocytopenische purpura
Estonian	Rekombinantne megakarüopoeesi stimuleeriv proteiin	Idiopaatilise trombotsütopeenilise purpura ravi
Finnish	Rekombinantti megakaryopoieesia stimuloiva proteiini	Idiopaattisen trombosytopeenisen purppuran hoito
French	Protéine recombinante stimulant la mégacaryopoïèse	Traitement du purpura thrombopénique idiopathique
German	Rekombinantes Megakaryopoese-stimulierendes Protein	Behandlung der idiopathischen thrombozytopenischen Purpura
Greek	Ανασυνδυασμένη πρωτεΐνη που διεγείρει την παραγωγή μεγακαρυοκυττάρων	Θεραπεία της Ιδιοπαθούς Θρομβοπενικής Πορφύρας.
Hungarian	Rekombináns megakaryocyta képződést serkentő fehérje	Idiopathiás thrombocytopeniás purpura kezelése
Italian	Proteina ricombinante stimolante la megacariopoiesi	Trattamento della Porpora Trombocitopenica idiomatica
Latvian	Rekombinants megakariopoēzi stimulējošs proteīns	Idiopātiskās trombocitopēniskās purpuras ārstēšanai
Lithuanian	Rekombinantinis megakariopoezę stimuliuojantis baltymas	Idiopatinės trombocitopeninės purpuros gydymas
Polish	Rekombinowane białko stymulujące megakariocytopoezę	Leczenie idiopatycznej plamicy małopłytkowej
Portuguese	Proteína recombinante estimuladora da megacariocitopoiese	Tratamento da Púrpura Trombocitopénica Idiopática
Slovak	Rekombinantný megakaryopoézu stimulujúci proteín	Liečba idiopatickej trombocytopenickej purpury
Slovenian	Rekombinantni megakariopoezo stimulirajoči protein	Zdravljenje idiopatske trombocitopenične purpure
Spanish	Proteína recombinante estimuladora de la megacariopoyesis	Tratamiento de la púrpura trombocitopénica idiopática
Swedish	Rekombinant megakaryopoesstimulerande protein	Behandling av idiopatiisk trombocytopen purpura
Norwegian	Rekombinant megakaryopoese-stimulerende protein	Behandling av idiopatisk trombocytopenisk purpura (ITP)
Icelandic	Raðbrigða megakaryopoiesis örvandi prótein	Til meðferðar við frumkomnum blóðflagnafæðarpurpura