



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation eltrombopag olamine for the treatment of idiopathic thrombocytopenic purpura

**Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in January 2012 on request of the sponsor.**

On 3 August 2007, orphan designation (EU/3/07/467) was granted by the European Commission to GlaxoSmithKline Research & Development Limited, United Kingdom, for eltrombopag olamine for the treatment of idiopathic thrombocytopenic purpura (ITP).

The sponsorship was transferred to GlaxoSmithKline Trading Services Limited, Ireland, in August 2008.

### What is idiopathic thrombocytopenic purpura?

Thrombocytopenic purpura 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. ITP is called "idiopathic" since the reason why the body starts reacting against its own cells is unknown. As a result, there will be fewer platelets present in the blood (thrombocytopenia), resulting in spontaneous bleeding and bruising of the skin in purple spots (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; it can vary from just small skin spots (petechiae) that occur after small injuries to spontaneous blood losses (haemorrhages) from the nose, in the gut or in the brain, 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 is equivalent to a total of between 50,000 and 199,000 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).

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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).



## 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 of spleen removal (splenectomy), or medicines. Splenectomy is performed in order to limit the destruction of the platelets, since the spleen is the most important organ where old platelets are removed from the blood. 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.

Eltrombopag olamine could be of potential significant benefit for the treatment of idiopathic thrombocytopenic purpura because it may act in a different way to 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.

## 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 develop into platelets through several cell divisions. Eltrombopag olamine 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 eltrombopag olamine 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.

At the time of submission of the application, eltrombopag olamine was not authorised anywhere in the world for idiopathic thrombocytopenic purpura, nor 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 27 June 2007 recommending the granting of this designation.

Update: Eltrombopag olamine (Revolade) has been authorised in the EU since 11 March 2010 for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second line treatment for adult non-splenectomised patients where surgery is contraindicated.

For more information on Revolade,

see: <http://www.ema.europa.eu/humandocs/Humans/EPAR/revolade/revolade.htm>

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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 European Union) 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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## Translations of the active ingredient and indication in all official EU languages<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Eltrombopag olamine	Treatment of idiopathic thrombocytopenic purpura
Bulgarian	Елтромбопаг оламин	Лечение на идиопатична тромбоцитопенична пурпура
Czech	Eltrombopag olamin	Léčba idiopatické trombocytopenické purpury
Danish	Eltrombopagolamin	Behandling af idiopatisk trombocytopenisk purpura
Dutch	Eltrombopag olamine	Behandeling van ideopathische trombocytopenische purpura
Estonian	Eltrombopagolamiin	Idiopaatilise trombotsütopeenilise purpura ravi
Finnish	Eltrombopagiolamiini	Idiopaattisen trombosytopeenisen purppuran hoito
French	Eltrombopag olamine	Traitement du purpura thrombopénique idiopathique
German	Eltrombopag Olatin	Behandlung der idiopathischen thrombozytopenischen Purpura
Greek	ολαμίνη eltrombopag	Θεραπεία της Ιδιοπαθούς Θρομβοπενικής Πορφύρας.
Hungarian	Eltrombopag olamin	Idiopathiás trombocytopeniás purpura kezelése
Italian	Eltrombopag olamina	Trattamento della Porpora trombocitopenica idiopatica
Latvian	Eltrombopaga olamīns	Idiopātiskās trombocitopēniskās purpuras ārstēšana
Lithuanian	Eltrombopago olaminas	Idiopatines trombocitopenines purpuros gydymas
Maltese	Eltrombopag olamine	Kura tal-purpura trombocitopenika idjopatika
Polish	Olatinian eltrombopagu	Leczenie idiopatycznej plamicy małopłytkowej
Portuguese	Eltrombopag olamina	Tratamento da Púrpura Trombocitopénica Idiopática
Romanian	Eltrombopag olamină	Tratamentul purpurei trombocitopenice idiopatice
Slovak	Eltrombopág olamín	Liečba idiopatickej trombocytopenickej purpury
Slovenian	Eltrombopag olamin	Zdravljenje idiopatske trombocitopenične purpure
Spanish	Eltrombopag Olamina	Tratamiento de la púrpura trombocitopénica idiopática
Swedish	Eltrombopagolamin	Behandling av idiopatisk trombocytopen purpura
Norwegian	Eltrombopagolamin	Behandling av idiopatisk trombocytopenisk purpura
Icelandic	Eltrombópag olamín	Til meðferðar við frumkomnum blóðflagnafæðarpurpura

<sup>1</sup> At the time of designation