



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

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## **COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

### **PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF acetylsalicylic acid for the treatment of polycythemia vera**

On 29 July 2004, orphan designation (EU/3/04/208) was granted by the European Commission to Bayer Vital GmbH, Germany, for acetylsalicylic acid for the treatment of polycythemia vera.

#### **What is polycythemia vera?**

Polycythemia vera is a disorder characterized by excessive and uncontrolled production of red blood cells, but also white blood cells and platelets, in the bone marrow. This feature of "too many blood cells" results in unique symptoms and complications. Persons with polycythemia vera are at higher risk of clotting or bleeding problems related to the resulting increased blood volume and viscosity (thickness). Symptoms caused by the elevated cell numbers such as headache, dizziness, visual disturbances, inability to concentrate and paresthesia (numbness) are present initially in 30 to 50% of patients. Affected persons typically develop an enlarged liver and spleen and may present with itching skin and gout. The increased number of cells in the blood can lead to formation of blood clots which impair the blood circulation and can result in necrosis of organs. Polycythemia vera is a chronically debilitating and life-threatening condition.

#### **What are the methods of treatment available?**

Other medicinal products were authorised for the treatment of polycythemia vera within the Community at the time of submission of the application for orphan drug designation.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product might be of potential significant benefit for the treatment of polycythemia vera, particularly in terms of preventing thrombosis (blood clot formation). The 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\*?**

According to the information provided by the sponsor, polycythemia vera was considered to affect about 92,000 to 138,000 persons in the European Union.

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

Platelets are blood cells taking part in the formation of blood clots. These blood components are for example, fundamental in the rapid formation of the clot to stop the bleeding in injuries. Acetylsalicylic acid (Aspirin) is known to interfere with this platelet function by inhibiting the synthesis of a substance called thromboxan that stimulates the formation of blood clots. So, in polycythemia patients acetylsalicylic acid (Aspirin) would reduce the risk of clot formation.

**What is the stage of development of this medicinal product?**

At the time of submission of the application for orphan designation, clinical trials in patients with polycythemia vera were completed.

Acetylsalicylic acid was not marketed anywhere worldwide for polycythemia vera 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 16 June 2004 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.

**For more information:**

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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 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**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	Acetylsalicylic acid	Treatment of polycythemia vera
Czech	Acetylsalicylová kyselina	Léčba polycythemia vera
Danish	Acetylsalicylsyre	Behandling af polycythaemia vera
Dutch	Acetylsalicylzuur	Behandeling van polycythaemia vera
Estonian	Atsetüülsalitsüülhape	Polycythemia vera ravi.
Finnish	Asetyylisalisyylihappo	Polysytomia veran hoitoon
French	Acide-acetylsalicylique	traitement de la Polycythaemia vera
German	Acetylsalicylsäure	Behandlung von Polycythemia vera
Greek	Ακετυλοσαλικυλικό οξύ	Θεραπεία της αληθούς πολυκυτταραιμίας, (Polycythaemia vera)
Hungarian	Acetilszalicilsav	Polycythaemia vera kezelésére
Italian	Acido acetilsalicilico	Terapia della policitemia vera
Latvian	Acetilsalicilskābe	Polycythemia vera ārstēšanai
Lithuanian	Acetilsalicilo rūgštis	Tikrosios policitemijos (Polycythemia vera) gydymas
Maltese	Ācidu acetilsalisiliku	Għall-kura tal-Policitemija vera
Polish	Kwas acetylosalicylowy	Leczenie czerwienicy prawdziwej
Portuguese	Ácido acetilsalicílico	Tratamento da policitemia vera
Slovak	Kyselina acetylsalicylová	Liečba pravej polycytémie
Slovenian	Acetilsalicilna kislina	Zdravljenje prave policitemije
Spanish	Ácido acetilsalicílico	Tratamiento de la policitemia vera
Swedish	Acetylsalicylsyra	Behandling av polycytomia vera
Norwegian	Acetylsalisylsyre	Behandling av polycythemia vera
Icelandic	Acetylsalicylsýra eða aspirín	Rauðkornamergð, rauðblæði, sjúklegt ástand er kemur fram í óeðlilegum fjölda rauðra blóðkorna