



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 artesunate for the treatment of malaria**

On 20 February 2007, orphan designation (EU/3/07/430) was granted by the European Commission to ACE Pharmaceuticals BV, The Netherlands, for artesunate for the treatment of malaria.

#### **What is malaria?**

Malaria is an infectious disease caused by *Plasmodium* parasites. There are four species of *Plasmodium* that may infect humans: *P. vivax*, *P. malariae*, *P. ovale* and *P. falciparum*. Malaria is carried from person to person by infected *Anopheles* mosquitoes, and transmission occurs mostly in tropical and subtropical regions including parts of the Americas, Asia, and Africa. In Europe, malaria is mainly encountered in travellers returning from these areas. Following a bite from an infected mosquito, the parasite goes to the liver. When the parasite then enters the bloodstream it infects and destroys red blood cells.

Fever is the major symptom of malaria. The disease must always be suspected when fever occurs during or after travel to an infected area. In addition, flu-like symptoms such as chills, headaches, muscle-aches, and fatigue can occur. Malaria due to *P. falciparum* may progress rapidly causing seizures, coma, breathing and kidney problems. The disease is life-threatening, and active malaria infection with *P. falciparum* is a medical emergency.

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

Several medicinal products with anti-malarial activity were authorised for the condition in 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 artesunate might be of potential significant benefit for the treatment of malaria, mainly because it might improve survival in those suffering severe, life-threatening malaria. 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\* ?**

Based on the information provided by the sponsor and previous knowledge of the Committee, malaria was considered to affect approximately 0.3 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 15,000 persons.

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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.

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

Artesunate is a chemically modified derivative of the natural product artemisinin, which is found in sweet wormwood. It is proposed that it acts by damaging the membrane of the parasite, thus killing the parasite.

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

The effects of artesunate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, artesunate was not authorised in the European Union.

Orphan designation of intravenously administered artesunate had been granted in the United States for immediate treatment of malaria.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 January 2007 a positive opinion recommending the grant of the above-mentioned designation.

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:

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Patients' associations contact points: Not available

**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         | Artesunate               | Treatment of malaria      |
| Bulgarian       | Артесунат                | Лечение на малария        |
| Czech           | Artesunate               | Léčba malárie             |
| Danish          | Artesunate               | Behandling af malaria     |
| Dutch           | Artesunaat               | Behandeling van malaria   |
| Estonian        | Artesunaat               | Malaaria ravi             |
| Finnish         | Artesunaatti             | Malaria hoito             |
| French          | Artesunate               | Traitement du paludisme   |
| German          | Artesunate               | Behandlung der Malaria    |
| Greek           | Αρτεσουνικό              | Θεραπεία της ελονοσίας    |
| Hungarian       | Artesunat                | Malária kezelése          |
| Italian         | Artesunato               | Trattamento della malaria |
| Latvian         | Artesunāts               | Malārijas ārstēšana       |
| Lithuanian      | Artesunatas              | Maliarijos gydymas        |
| Maltese         | Artesunate               | Kura tal-malarja          |
| Polish          | Artesunat                | Leczenie malarii          |
| Portuguese      | Artesunate               | Tratamento de malária     |
| Romanian        | Artesunat                | Tratamentul malariei      |
| Slovak          | Artesunat                | Liečba malária            |
| Slovenian       | Artesunat                | Zdravljenje malarije      |
| Spanish         | Artesunate               | Tratamiento del paludismo |
| Swedish         | Artesunat                | Behandling av malaria     |
| Norwegian       | Artesunat                | Behandling av malaria     |
| Icelandic       | Artesúnat                | Til meðferðar á malaríu   |