



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

**PUBLIC SUMMARY OF
POSITIVE OPINION FOR ORPHAN DESIGNATION
OF
artesunate
for the treatment of malaria**

On 6 December 2007, orphan designation (EU/3/07/510) was granted by the European Commission to Sigma-tau Pharma UK, United Kingdom, 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 requiring hospitalization.

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 in total.

* 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?

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, clinical trials in patients with malaria were ongoing.

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 October 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.

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

Language	Active Ingredient	Indication
English	Artesunate	Treatment of malaria
Bulgarian	Артесунат	Лечение на малария
Czech	Artesunate	Léčba malárie
Danish	Artesunat	Behandling af malaria
Dutch	Artesunaat	Behandeling van malaria
Estonian	Artesunaat	Malaaria ravi
Finnish	Artesunaatti	Malarian hoito
French	Artesunate	Traitement du paludisme
German	Artesunat	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	Artesunato	Tratamento da 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