



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

14 October 2013
EMA/COMP/486932/2007 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Artesunate for the treatment of malaria

First publication	20 December 2007
Rev.1: administrative update	1 July 2008
Rev.2: transfer of sponsorship	8 May 2012
Rev.3: administrative update	14 October 2013
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

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.

The sponsorship was transferred to Sigma-Tau Industrie Farmaceutiche Riunite S.p.A., Italy, in April 2012.

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 is the estimated number of patients affected by the condition?

At the time of designation, malaria affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 people*, and is below the ceiling 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).

What treatments are 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.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

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 medicine?

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.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 recommending the granting of this designation.

*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. At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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 EU) 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:

Sigma-Tau Industrie Farmaceutiche Riunite S.p.A.
Viale Shakespeare 47
00144 Rome
Italy
Telephone: +39 06 913 93 657
Telefax: +39 06 911 66 976
E-mail: giulia.bruno@sigma-tau.it

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, 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	Artezunat	Zdravljenje malarije
Spanish	Artesunate	Tratamiento del paludismo
Swedish	Artesunat	Behandling av malaria
Norwegian	Artesunat	Behandling av malaria
Icelandic	Artesúnat	Til meðferðar á malaríu

¹ At the time of designation