



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Artesunate for the treatment of malaria

On 6 December 2012, orphan designation (EU/3/12/1079) was granted by the European Commission to Dafra Pharma International, Belgium, 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* parasites that may infect humans: *P. vivax*, *P. malariae*, *P. ovale* and *P. falciparum*. They are transmitted from person to person by the bite of infected *Anopheles* mosquitoes. Once in the body, the parasites multiply in the liver and then infect and destroy red blood cells.

Transmission mostly occurs in tropical and subtropical regions including parts of the Americas, Asia, and Africa. In Europe, malaria mainly affects travellers returning from these areas.

Fever is the major symptom of malaria and 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 commonly occur.

Malaria is a severe and life-threatening disease because of its complications such as cerebral malaria (a deep coma caused by infected red blood cells adhering to blood vessels in the brain), seizures (fits), and lung and kidney problems. Malaria due to *P. falciparum* is the most severe form and may rapidly lead to complications, especially in children and others with low immunity (protection) to the disease.

What is the estimated number of patients affected by the condition?

At the time of designation, malaria affected not more than 0.3 in 10,000 people per year in the European Union (EU)*. This is equivalent to a total of not more than 15,000 people per year, which is considered to be below the ceiling for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 506,300,000 (Eurostat 2011).



What treatments are available?

At the time of designation, several medicines were authorised in the EU to treat malaria, including products containing artemisinin derivatives such as artesunate to be given by mouth. Quinine is the only medicine authorised in the EU that is given into a vein.

The sponsor has provided sufficient information to show that artesunate might be of significant benefit for patients with malaria, mainly because it might improve survival in those suffering severe, life-threatening malaria. The medicine given into a vein has been shown in large studies to be more effective than quinine at improving the survival of patients with severe 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 derivative of the naturally occurring substance artemisinin. It is expected to be given by injection into a vein or muscle. Its exact mode of action is not clear, but once it has entered blood cells infected by the parasite, it is expected to interact with human blood components to release substances that damage the membrane of the malaria parasite, thereby killing it.

What is the stage of development of this medicine?

As artesunate is a well-known anti-malarial agent, the sponsor has provided data in experimental models from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, clinical trials with artesunate in patients with malaria had been completed.

At the time of submission, artesunate was not authorised anywhere in the EU for malaria. Orphan designation of artesunate had been granted in the EU and the United States of America for the treatment of malaria.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 November 2012 recommending the granting of this designation.

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:

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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 substance	Indication
English	Artesunate	Treatment of malaria
Bulgarian	Артесунат	Лечение на малария
Czech	Artesunat	Léčba malárie
Danish	Artesunat	Behandling af malaria
Dutch	Artesunaat	Behandeling van malaria
Estonian	Artesunaat	Malaaria ravi
Finnish	Artesunaatti	Malarian hoito
French	Artésunate	Traitement du paludisme
German	Artesunat	Behandlung der Malaria
Greek	Αρτεσουνικό	Θεραπεία της ελονοσίας
Hungarian	Arteszunat	Malária kezelése
Italian	Artesunato	Trattamento della malaria
Latvian	Artezunāts	Malārijas ārstēšana
Lithuanian	Artesunatas	Maliarijos gydymas
Maltese	Artesunate	Kura tal-malarja
Polish	Artezunat	Leczenie malarii
Portuguese	Artesunato	Tratamento da malária
Romanian	Artesunat	Tratamentul malariei
Slovak	Artesunát	Liečba malárie
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