



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Public summary of opinion on orphan designation

### Artesunate for the treatment of malaria

On 28 February 2020, orphan designation EU/3/20/2251 was granted by the European Commission to YES Pharmaceutical Development Services GmbH, Germany, for artesunate for the treatment of malaria.

#### What is malaria?

Malaria is an infection caused by Plasmodium parasites. The main species of Plasmodium parasites that infect humans are: *P. falciparum* and *P. vivax*, and less frequently, *P. malariae* and *P. ovale*. They spread through the bite of infected Anopheles mosquitoes. Once in the body, the parasites multiply in the liver and then infect and destroy red blood cells.

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

Fever, the major symptom of malaria, can occur during or after travel to an infected area. In addition, flu-like symptoms such as chills, headaches, muscle aches and fatigue commonly occur.

Malaria caused by *P. falciparum* is a severe and life-threatening disease because of its complications such as cerebral malaria (a deep coma caused by infected red blood cells in the brain), seizures (fits), and lung and kidney problems. It is especially dangerous during pregnancy, in children and in people with low immunity (protection) against the disease.

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

At the time of designation, malaria affected approximately 0.12 in 10,000 people in the European Union (EU). This was equivalent to a total of around 6,000 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).

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\*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, Norway, Iceland and Liechtenstein. This represents a population of 519,200,000 (Eurostat 2020).



## **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. Eurartesim, which contains the active substances piperazine tetraphosphate and artemimol (an artemisinin derivative) was authorised across the EU. Quinine is the only medicine authorised in the EU that can be given by injection into a vein.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with severe malaria who need treatment by injection into a vein. Studies have shown that artesunate seems to be more effective than quinine. 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. Most malaria medicines are only able to kill specific life-cycle stages of the malaria parasite but artemisinin derivatives act on all stages and are therefore expected to be more effective than existing medicines. Artesunate is expected to be given by injection into a vein or muscle. The exact way it works is not clear, but once it enters infected red blood cells, it causes the release of substances that damage proteins in the malaria parasite, thereby killing it.

## **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 with artesunate in patients with malaria had finished.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 22 January 2020, recommending the granting of this designation.

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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:

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Artesunate	Treatment of malaria
Bulgarian	Артесунат	Лечение на малария
Croatian	Artesunat	Liječenje malarije
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	Arteszumat	Malária kezelése
Italian	Artesunato	Trattamento della malaria
Latvian	Artezunāts	Malārijas ārstēšana
Lithuanian	Artesunatas	Maliarijos gydymas
Maltese	Artesunate	Kura tal-malarja
Polish	Artezumat	Leczenie malarii
Portuguese	Artesunato	Tratamento da malária
Romanian	Artesunat	Tratamentul malariei
Slovak	Artesunát	Liečba malárie
Slovenian	Artezumat	Zdravljenje malarije
Spanish	Artesunate	Tratamiento del paludismo
Swedish	Artesunat	Behandling av malaria
Norwegian	Artesunat	Behandling av malaria
Icelandic	Artesúnat	Til meðferðar á malaríu

<sup>1</sup> At the time of designation