



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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EMA/4539/2017  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Hydroxychloroquine for treatment of antiphospholipid syndrome

On 12 January 2017, orphan designation (EU/3/16/1820) was granted by the European Commission to Centre Hospitalier Universitaire d'Angers, France, for hydroxychloroquine for the treatment of antiphospholipid syndrome.

#### What is antiphospholipid syndrome?

Antiphospholipid syndrome (APS) is a clotting disorder in which patients are at greater risk of developing conditions such as deep vein thrombosis (a blood clot that usually develops in the leg) and arterial thrombosis (a clot in an artery), which can cause a stroke or heart attack. In women, APS is a known cause of recurring miscarriages because of blood clots in vessels supplying the baby.

APS is caused by the body's own immune (defence) system producing antibodies (known as antiphospholipid antibodies), which are thought to interfere with the clotting process and make the blood more likely to clot.

APS is a long-term debilitating and life-threatening disease because of the risk of heart attack, stroke and miscarriages.

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

At the time of designation, APS affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 people<sup>\*</sup>, 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?

At the time of designation, there were no treatments authorised in the EU for antiphospholipid syndrome. Anticoagulants (e.g. warfarin) and platelet aggregation inhibitors (e.g. heparin) were used to prevent blood clots.

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<sup>\*</sup>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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).



## How is this medicine expected to work?

Hydroxychloroquine is expected to work in APS by stopping platelets clumping together, reducing production of antiphospholipid antibodies and lowering cholesterol levels. Together, these effects are expected to reduce the risk of blood clots.

## What is the stage of development of this medicine?

The effects of hydroxychloroquine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with hydroxychloroquine in patients with APS were ongoing.

At the time of submission, hydroxychloroquine was authorised in the EU for various immune system conditions including rheumatoid arthritis.

At the time of submission, hydroxychloroquine was not authorised anywhere in the EU for APS or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2016 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, on the medicine's [rare disease designations page](#).

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	Hydroxychloroquine	Treatment of antiphospholipid syndrome
Bulgarian	Хидрохисхлороквин	Лечение на антифосфолипиден синдром
Croatian	Hidroksiklorokin	Liječenje antifosfolipidnog sindroma
Czech	Hydroxychloroquin	Léčba antifosfolipidového syndromu
Danish	Hydroxychloroquin	Behandling af antifosfolipid syndrom
Dutch	Hydroxychloroquine	Behandeling van antifosfolipidensyndroom
Estonian	Hüdroksüklorokviini	Antifosfolipiidsündroomi ravi
Finnish	Hydroksiklorokiin	Fosfolipidivasta-ainesyndrooman hoito
French	Hydroxychloroquine	Traitement du syndrome des antiphospholipides
German	Hydroxychloroquin	Behandlung des Anti-Phospholipid-Syndroms
Greek	Υδροξυχλωροκίνη	Θεραπεία του αντιφωσφολιπιδικού συνδρόμου
Hungarian	Hidroxiklorokin	Antifoszfolipid szindróma kezelése
Italian	Idrossiclorochina	Trattamento della sindrome da anticorpi antifosfolipidi
Latvian	Hidroksihlorohīns	Antifosfolipīdu sindroma ārstēšana
Lithuanian	Hidroksichlorokvinas	Antifosfolipidinio sindromo gydymas
Maltese	Hydroxychloroquine	Kura tas-sindromu antifosfolipidu
Polish	Hydroksychlorokwina	Leczenie zespołu antyfosfolipidowego
Portuguese	Hidroxicloroquina	Tratamento da síndrome antifosfolípido
Romanian	Hidroxiclorochină	Tratamentul sindromului antifosfolipidic
Slovak	Hydroxychlorochín	Liečba antifosfolipidového syndrómu
Slovenian	Hidroksiklorokin	Zdravljenje antifosfolipidni sindrom
Spanish	Hidroxicloroquina	Tratamiento del síndrome antifosfólipido
Swedish	Hydroxiklorokin	Behandling av antifosfolipidsyndrom
Norwegian	Hydroksyklorokvine	Behandling av antifosfolipidsyndrom
Icelandic	Hýdroxýklórókínn	Meðferð andfósólípíðmótefna heilkenni

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