



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mepolizumab for the treatment of Churg-Strauss syndrome

On 12 March 2013, orphan designation (EU/3/13/1116) was granted by the European Commission to Glaxo Group Ltd, United Kingdom, for mepolizumab for the treatment of Churg-Strauss syndrome.

What is Churg-Strauss syndrome?

Churg-Strauss syndrome is a type of vasculitis (a chronic inflammation of small to medium-sized arteries and veins). The causes of the disease are not known but it is characterised by fever, hypereosinophilia (abnormally high production of a type of white blood cells called eosinophils) and granulomatosis (clumping of eosinophils into small granular lumps that result in inflammation). Other symptoms of the disease include asthma and sinus problems, numbness or pain in the extremities, and inflammation of organs, including the lungs, heart, kidneys and gut.

Churg-Strauss syndrome is a long-term debilitating and life-threatening condition due to the inflammation of the organs. The main causes of death are myocarditis (inflammation of the heart muscle) and myocardial infarction (heart attack) following coronary arteritis (inflammation of the coronary arteries).

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

At the time of designation, Churg-Strauss syndrome affected not more than 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 25,000 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?

At the time of orphan designation there were no satisfactory treatments authorised in the EU for Churg-Strauss syndrome. The symptoms of milder forms of the disease were usually treated with anti-inflammatory medicines called corticosteroids.

*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 509,000,000 (Eurostat 2013).



How is this medicine expected to work?

Mepolizumab is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific protein found in the body called interleukin-5 (IL-5). IL-5 promotes the production and growth of eosinophils. By attaching to it, mepolizumab is expected to stop it working, reducing the production and survival of eosinophils. As eosinophils are the cells that are involved in causing Churg-Strauss syndrome, this is expected to improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of mepolizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with mepolizumab in patients with Churg-Strauss syndrome were ongoing.

At the time of submission, mepolizumab was not authorised anywhere in the EU for Churg-Strauss syndrome. Orphan designation of mepolizumab had been granted in the United States of America for Churg-Strauss syndrome. Orphan designation had also been obtained in the EU for hypereosinophilic syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2013 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 ingredient	Indication
English	Mepolizumab	Treatment of Churg-Strauss Syndrome
Bulgarian	Меполизумаб	Лечение на синдром на Churg-Strauss
Czech	Mepolizumab	Pro léčbu Churg-Straussova syndromu
Danish	Mepolizumab	Behandling af Churg-Strauss syndrom
Dutch	Mepolizumab	Behandeling van Churg-Strauss Syndroom
Estonian	Mepolizumab	Churg-Straussi sündroomi ravi
Finnish	Mepolitsumabi	Churg-Strauss -oireyhtymän hoito.
French	Mépolizumab	Traitement du syndrome de Churg-Strauss
German	Mepolizumab	Zur Behandlung des Churg-Strauss- Syndroms
Greek	Μεπολιζουμάμπη	Για τη θεραπεία του Συνδρόμου Churg-Strauss
Hungarian	Mepolizumab	Churg-Strauss szindróma kezelése
Italian	Mepolizumab	Trattamento della sindrome di Churg-Strauss
Latvian	Mepolizumabs	Churg-Strauss sindroma ārstēšanai
Lithuanian	Mepolizumabas	Churg-Strauss sindromo gydymas
Maltese	Mepolizumab	Kura tas-sindrome ta' Churg-Strauss
Polish	Mepolizumab	Leczenie zespołu Churg-Strauss
Portuguese	Mepolizumab	Para o tratamento do síndrome de Churg-Strauss
Romanian	Mepolizumab	Pentru tratamentul sindromului Churg-Strauss
Slovak	Mepolizumab	Na liečbu Churgovho-Straussovej syndrómu
Slovenian	Mepolizumab	Za zdravljenje Churg-Straussovega sindroma
Spanish	Mepolizumab	Tratamiento del síndrome de Churg-Strauss
Swedish	Mepolizumab	För behandling av Churg-Strauss syndrom
Norwegian	Mepolizumab	Behandling av Churg-Strauss syndrom
Icelandic	Mepólízúmab	Til meðferðar á Churg-Strauss heilkenni

¹ At the time of designation