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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Gevokizumab for the treatment of chronic non-infectious uveitis

On 12 March 2013, orphan designation (EU/3/13/1111) was granted by the European Commission to Les Laboratoires Servier, France, for gevokizumab for the treatment of chronic non-infectious uveitis.

What is chronic non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye. The inflammation can affect one or both eyes, and may cause discomfort, pain, and blurring of vision. Non-infectious uveitis is usually caused by the body's immune system (the body's natural defences) attacking normal tissue and not by an infection.

Chronic (long-term) non-infectious uveitis is a long-term debilitating disease because it may lead to partial or complete loss of vision (blindness).

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

At the time of designation, chronic non-infectious uveitis affected approximately 3.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 168,000 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 designation, several medicines were authorised in the EU for the treatment of non-infectious uveitis. The first treatment option was corticosteroids, which were used to reduce the inflammation by lowering the activity of the immune system. Other immunosuppressant agents such as ciclosporin were also authorised for use in chronic non-infectious uveitis.

The sponsor has provided sufficient information to show that gevokizumab might be of significant benefit for patients with chronic non-infectious uveitis because early studies suggest that it might reduce the loss of vision and its use may result in a reduction in the use of corticosteroids. These

*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).

assumptions 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein in the body called interleukin-1 beta. Interleukin-1 beta is involved in the process of inflammation and is thought to play a role in chronic non-infectious uveitis. By attaching to interleukin-1 beta, this medicine is expected to block its activity, helping to relieve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of gevokizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with gevokizumab in patients with chronic non-infectious uveitis were ongoing.

At the time of submission, gevokizumab was not authorised anywhere in the EU for chronic non-infectious uveitis. Orphan designation of gevokizumab had been granted in the United States of America for the treatment of non-infectious intermediate, posterior or pan uveitis, or chronic non-infectious anterior uveitis.

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:

Les Laboratoires Servier
50, rue Carnot
92284 Suresnes Cedex
France
Telephone: +33 155 7234 93
Telefax: +33 155 7254 12
E-mail: vanessa.daros@fr.netgrs.com

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	Gevokizumab	Treatment of chronic non-infectious uveitis
Bulgarian	Гевокизумаб	Лечение на хроничен неинфекциозен увеит
Czech	Gevokizumab	Léčba chronické neinfekční uveitidy
Danish	Gevokizumab	Behandling af kronisk ikke-infektiøs uveitis
Dutch	Gevokizumab	Behandeling van chronische niet-infectieuze uveïtis
Estonian	Gevokizumab	Kroonilise mitteinfektsioosse uveidi ravi
Finnish	Gevokitsumabi	Kroonisen, ei-infektioperäisen uveiitin hoito
French	Gévokizumab	Traitement de l'uvéïte chronique non infectieuse
German	Gevokizumab	Behandlung der chronischen nicht-infektiösen Uveitis
Greek	Γκεβοκίζουμάμπη	Θεραπεία της χρόνιας μη μολυσματικής ραγοειδίτιδας
Hungarian	Gevokizumab	Krónikus, nem fertőzőes eredetű uveitis kezelése
Italian	Gevokizumab	Trattamento dell'uveite cronica non infettiva
Latvian	Gevokizumabs	Hroniskā neinfekciozā uveīta ārstēšana
Lithuanian	Gevokizumabas	Lėtinio neinfekcinio uveito gydymas
Maltese	Gevokizumab	Kura ta' l-uvejite kronika u mhux infettiva
Polish	Gewokizumab	Leczenie przewlekłego niezakaźnego zapalenia błony naczyniowej oka
Portuguese	Gevokizumab	Tratamento da uveíte crónica não infecciosa
Romanian	Gevokizumab	Tratamentul uveitei non-infecțioase cronice
Slovak	Gevokizumab	Liečba chronickej neinfekčnej uveitídy
Slovenian	Gevokizumab	Zdravljenje kroničnega neinfekcijskega uveitisa
Spanish	Gevokizumab	El tratamiento de la uveitis crónica no infecciosa
Swedish	Gevokizumab	Behandling av kronisk icke infektiös uveit
Norwegian	Gevokizumab	Behandling av kronisk ikke-infeksiøs uveitt
Icelandic	Gevókízúmab	Til meðferðar á augnæðahjúpsbólgu sem ekki stafar af sýkingu

¹ At the time of designation