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EMA/COMP/436241/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Gevokizumab for the treatment of Schnitzler syndrome

On 22 August 2014, orphan designation (EU/3/14/1311) was granted by the European Commission to Les Laboratoires Servier, France, for gevokizumab for the treatment of Schnitzler syndrome.

What is Schnitzler syndrome?

Schnitzler syndrome is a disease characterised by long-term urticaria (hives), monoclonal gammopathy (abnormal amounts of certain proteins in the blood), recurrent fever, bone and joint pain, and swollen lymph nodes. The disease usually appears during adulthood. Its cause is unclear.

Schnitzler syndrome is a long-term debilitating and life-threatening disease as it may lead to complications, including severe anaemia (low red blood cell counts), amyloidosis (build-up of abnormal protein deposits) and lymphoproliferative disorders (where white blood cells are produced in excessive amounts).

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

At the time of designation, Schnitzler syndrome affected approximately 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of around 1,000 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, no satisfactory methods were authorised for the treatment of Schnitzler syndrome. Corticosteroids and anti-inflammatory medicines known as NSAIDs were used to treat the symptoms of the disease. Anakinra (a medicine that works by blocking the receptor for a protein in the body called interleukin-1 beta) was also used.

*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 511,100,000 (Eurostat 2014).

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How is this medicine expected to work?

Gevokizumab is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to the protein interleukin-1 beta. Interleukin-1 beta is involved in the process of inflammation and is thought to play a role in Schnitzler syndrome. 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 Schnitzler syndrome were ongoing.

At the time of submission, gevokizumab was not authorised anywhere in the EU for Schnitzler syndrome 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 10 July 2014 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Gevokizumab	Treatment of Schnitzler syndrome
Bulgarian	Гевокизумаб	Лечение на синдром на Шницлер
Croatian	Gevokizumab	Liječenje Schnitzlerovog sindroma
Czech	Gevokizumab	Léčba Schnitzlerova syndromu
Danish	Gevokizumab	Behandling af Schnitzlers syndrom
Dutch	Gevokizumab	Behandeling van het syndroom van Schnitzler
Estonian	Gevokizumab	Schnitzler´i sündroomi ravi
Finnish	Gevokitsumabi	Schnitzlerin oireyhtymän hoito
French	Gévokizumab	Traitement du syndrome de Schnitzler
German	Gevokizumab	Behandlung des Schnitzler-Syndroms
Greek	Γκεβοκιζουμάπμη	Θεραπεία του συνδρόμου Schnitzler
Hungarian	Gevokizumab	Schnitzler-szindróma kezelése
Italian	Gevokizumab	Trattamento della sindrome di Schnitzler
Latvian	Gevokizumabs	Šniclera sindroma ārstēšana
Lithuanian	Gevokizumabas	*Schnitzler* sindromo gydymas
Maltese	Gevokizumab	Kura tas-sindrome ta' Schnitzler
Polish	Gewokizumab	Leczenie zespołu Schnitzlera
Portuguese	Gevokizumab	Tratamento da síndrome de Schnitzler
Romanian	Gevokizumab	Tratamentul sindromului Schnitzler
Slovak	Gevokizumab	Liečba Schnitzlerovho syndrómu
Slovenian	Gevokizumab	Zdravljenje Schnitzlerjevega sindroma
Spanish	Gevokizumab	Tratamiento del síndrome de Schnitzler
Swedish	Gevokizumab	Behandling av Schnitzlers syndrom
Norwegian	Gevokizumab	Behandling av Schnitzlers syndrom
Icelandic	Gevókízúmab	Meðferð við Schnitzler heilkenni

[1] At the time of designation