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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mepolizumab for the treatment of hypereosinophilic syndrome

On 29 July 2004, orphan designation (EU/3/04/213) was granted by the European Commission to SmithKline Beecham plc, United Kingdom, for mepolizumab for the treatment of hypereosinophilic syndrome.

The sponsorship was transferred to Glaxo Group Limited, United Kingdom, in September 2008.

What is hypereosinophilic syndrome?

Hypereosinophilic syndrome is a type of leukaemia (chronic eosinophilic leukaemia) for which it is impossible to find the origin of the cancer cells. It is a disease in which cancer cells are found in the blood, the bone marrow and in tissues. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells called "blasts" that mature into several different types of blood cells that have specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. White blood cells fight infection. Platelets make the blood clot. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukaemias. In the hypereosinophilic syndrome, blasts that are developing into white blood cells called "eosinophils" are affected. The eosinophils become too numerous and are then found in the bone marrow, blood and in other tissues such as the heart, lungs, nerves and skin. The excess of eosinophils in tissues can cause disturbances in the function and thereby damage the affected organs. The hypereosinophilic syndrome is life-threatening.

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

At the time of designation, hypereosinophilic syndrome affected approximately 0.15 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 7,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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What treatments are available?

No satisfactory methods exist that were authorised at the time of application.

How is this medicine expected to work?

Different substances produced in the body as interleukin 5, interleukin 3 and granulocyte-monocyte colony stimulating factor (GMCSF) regulate eosinophilic cells production. Antibodies are proteins in the body that target specific shapes (receptors) on the surface of foreign bodies, such as bacteria or cancer cells. Mepolizumab is a human protein that is able to recognise and bind to the receptor of interleukin 5, present on the surface of the eosinophilic cells. It is expected that mepolizumab, by binding to the eosinophilic cells, will prohibit interleukin 5 to interact with its receptor. Thereby interleukin 5 will not be able to stimulate the production of eosinophils anymore. This could then lead to a decrease of the number of eosinophils in blood.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no clinical trials in patients with hypereosinophilic syndrome were initiated.

Mepolizumab was not marketed anywhere worldwide for hypereosinophilic syndrome. Orphan designation of mepolizumab was granted in the United States for treatment of hypereosinophilic syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 June 2004 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Mepolizumab	Treatment of hypereosinophilic syndrome
Bulgarian	Меполизумаб	Лечение на хипереозинофилен синдром
Czech	Mepolizumab	Léčba hypereozinofilního syndromu
Danish	Mepolizumab	Behandling af hypereosinofilt syndrom
Dutch	Mepolizumab	Behandeling van hypereosinofiel syndroom
Estonian	Mepolizumab	Hüpereosinofiilse sündroomi ravi..
Finnish	Mepolitsumabi	Hypereosinofiilisen oireyhtymän hoiton.
French	Mépolizumab	Traitement du syndrome d'hyperéosinophilie
German	Mepolizumab	Behandlung des hypereosinophilen Syndroms
Greek	Mepolizumab	Θεραπεία του υπερηωζινοφιλικού συνδρόμου
Hungarian	Mepolizumab	Hypereosinophiliás szindróma kezelése
Italian	Mepolizumab	Trattamento della sindrome ipereosinofilica
Latvian	Mepolizumabs	Hipereozinofīlijas sindroma ārstēšanai.
Lithuanian	Mepolizumabas	Hipereozinofilinio sindromo gydymas
Maltese	Mepolizumab	Trattament ta' hypereosinophilic syndrome
Polish	Mepolizumab	Leczenie zespołu hipereozynofilii
Portuguese	Mepolizumab	Tratamento do síndrome hipereosinofilico
Romanian	Mepolizumab	Tratamentul sindromului hipereosinofilic
Slovak	Mepolizumab	Liečba hypereozinofilného syndrómu
Slovenian	Mepolizumab	Zdravljenje hipereozinofilnega sindroma
Spanish	Mepolizumab	Tratamiento del síndrome hipereosinofílico
Swedish	Mepolizumab	Behandling av hypereosinofilt syndrom
Norwegian	Mepolizumab	Behandling av hypereosinofilt syndrom
Icelandic	Mepolizumab	Meðferðar heilkennis af völdum fjölgunar eósínófíla

[1] At the time of transfer of sponsorship