



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

monoclonal antibody to human interleukin-6 for the treatment of post-transplant lymphoproliferative disorders

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in January 2010 on request of the sponsor.

On 18 December 2002, orphan designation (EU/3/02/125) was granted by the European Commission to OPI, France, for monoclonal antibody to human interleukin-6 for the treatment of post-transplant lymphoproliferative disorders.

OPI changed its name to EUSA Pharma SAS in February 2008.

What are the post-transplantation lympho-proliferative disorders?

The lymphatic system consists of the tissues and organs that produce, store, and carry white blood cells. These cells fight infection and other diseases. White blood cells also attack cells that look foreign. This may happen for example after an organ transplant, where it may lead to a rejection of the transplanted organ. Therefore, organ transplant requires therapy to inhibit the white blood cells. However, in response to such therapy, cancer may develop from white blood cells. This is then called "post-transplant lymphoproliferative disorders" (PTLD). It is often similar to other lymphatic cancers (lymphomas). PTLD can be life-threatening.

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

At the time of designation, post-transplant lymphoproliferative disorders affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 11,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.



What treatments are available?

There exist authorised treatments for certain lymphomas in the Community. These can sometimes be used in the treatment of PTLD. Changing the treatment used to prevent the rejection may sometimes stop the development of the disease. However, this may expose the patient to a risk of rejection of the transplanted organ. Monoclonal antibody to human interleukin-6 may be of potential significant benefit for the treatment for the condition.

How is this medicine expected to work?

Interleukin-6 (IL6) is a small protein produced by numerous types of cells. Patients with PTLD produce abnormally high levels of IL6. IL6 may help the proliferation of cancer cells in patients with PTLD. The antibody is expected to target IL6, preventing this proliferation.

What is the stage of development of this medicine?

The evaluation of the effects of monoclonal antibody to interleukin-6 on post-transplant lymphoproliferative disorders in experimental models is ongoing, and clinical trials in patients with patients affected by PTLD were planned.

Monoclonal antibody to human interleukin-6 had not been marketed anywhere worldwide for prevention of PTLD or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 15 November 2002 a positive opinion recommending the grant of the above-mentioned 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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