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EMA/COMP/471332/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pralatrexate for the treatment of Hodgkin's lymphoma

On 1 October 2010, orphan designation (EU/3/10/795) was granted by the European Commission to Allos Therapeutics Limited, United Kingdom, for pralatrexate for the treatment of Hodgkin's lymphoma.

What is Hodgkin's lymphoma?

Hodgkin's lymphoma is a type of cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. Because lymph nodes are found throughout the body, the cancer can begin in almost any part of the body. In Hodgkin's lymphoma, white blood cells in the lymphatic system multiply too quickly and live for too long. These cancer cells can spread through the lymphatic system to other lymph nodes or through the bloodstream to other organs such as the spleen, where they can form new tumours.

Many people with Hodgkin's lymphoma can be cured if the disease is found and treated early. However, despite the treatments available, Hodgkin's lymphoma remains a serious and life-threatening disease, mainly because it is associated with poor survival in patients whose disease does not respond to treatment or has come back after previous treatment.

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

At the time of designation, Hodgkin's lymphoma affected approximately 1.1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 56,000 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).

What treatments are available?

At the time of designation, several medicines were authorised for the treatment of Hodgkin's lymphoma in the EU. The main treatments for Hodgkin's lymphoma included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem cell transplantation was also used when the disease had not responded to treatment or had come back

*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 506,500,000 (Eurostat 2010).

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after treatment. This is a complex procedure where patients receive their own stem cells to help restore the bone marrow.

The sponsor has provided sufficient information to show that pralatrexate might be of significant benefit for patients with Hodgkin's lymphoma because early clinical studies show that it might improve the treatment of patients with this condition. This assumption 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?

Pralatrexate is an 'antimetabolite' medicine. In the body, it is expected to take the place of folic acid and attach to an enzyme called dihydrofolate reductase (DHFR). DHFR is necessary for the production of new DNA and proteins, which are required for cells to divide and multiply. By attaching to DHFR, pralatrexate is expected to block the enzyme's activity, inhibiting the growth of the cancer cells and eventually killing them.

What is the stage of development of this medicine?

The effects of pralatrexate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pralatrexate including patients with Hodgkin's lymphoma were ongoing.

At the time of submission, pralatrexate was not authorised anywhere in the EU for Hodgkin's lymphoma 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 16 July 2010 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pralatrexate	Treatment of Hodgkin's lymphoma
Bulgarian	Пралатрексат	Лечение на лимфом на Хочкин
Czech	Pralatrexat	Léčba Hodgkinova lymfomu
Danish	Pralatrexat	Behandling af Hodgkin lymfom
Dutch	Pralatrexate	Behandeling van Hodgkin lymfoom
Estonian	Pralatreksaat	Hodgkini lümfoomi ravi
Finnish	Pralatreksaatti	Hodgkinin lymfooman hoito
French	Pralatrexate	Traitement du lymphome de Hodgkin
German	Pralatrexat	Behandlung des Hodgkin-Lymphoms
Greek	Πραλατρεξάτη	Θεραπεία του λεμφώματος Hodgkin
Hungarian	Pralatrexat	Hodgkin lymphoma kezelése
Italian	Pralatrexato	Trattamento del linfoma di Hodgkin
Latvian	Pralatreksāts	Hodžkina limfomas ārstēšana
Lithuanian	Pralatreksatas	Hodžkino limfomos gydymas
Maltese	Pralatrexate	Kura tal-limfoma ta' Hodgkin
Polish	Pralatreksat	Leczenie chłoniaka Hodgkina (ziarnicy złośliwej)
Portuguese	Pralatrexato	Tratamento do linfoma de Hodgkin
Romanian	Pralatrexat	Tratamentul limfomului Hodgkin
Slovak	Pralatrexát	Liečba lymfómu Hodgkinovho typu
Slovenian	Pralatreksat	Zdravljenje Hodgkinovega limfoma
Spanish	Pralatrexato	Tratamiento del linfoma de Hodgkin
Swedish	Pralatrexat	Behandling av Hodgkin lymfom
Norwegian	Pralatreksat	Behandling av Hodgkin-lymfom
Icelandic	Pralatrexat	Meðferð við Hodgkins sjúkdómi

[1] At the time of designation