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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Temsirolimus for the treatment of mantle cell lymphoma

On 6 November 2006, orphan designation (EU/3/06/420) was granted by the European Commission to Wyeth Europa Limited, United Kingdom, for temsirolimus for the treatment of mantle cell lymphoma.

The sponsorship was transferred to Pfizer Limited, United Kingdom, in September 2011.

What is mantle cell lymphoma?

Mantle cell lymphoma belongs to the group of diseases called “non-Hodgkin lymphomas”. Lymphoma is a type of cancer that originates from the lymphatic system. The lymphatic system is part of the immune system: the body's natural defence against infection and disease. It is a complex system made up of organs such as the bone marrow (the spongy tissue inside the large bones in the body), the thymus and the spleen, and a network of lymph nodes throughout the body that are connected by lymphatic vessels. Normally, the lymphatic cells grow in a controlled manner. However, in lymphoma a lymphatic cell continues to grow and divides in an uncontrolled manner, developing into a tumour. Lymphoma cells generally grow in lymph nodes. There are several different types of lymphoma depending on the type of lymphatic cancer cells. In mantle cell lymphoma, the cancer cells are related to the blood cells called B-lymphocytes. Mantle cell lymphoma is a life-threatening condition.

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

At the time of designation mantle cell lymphoma affected approximately 0.4 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 18,000 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).

What treatments are available?

Treatment of mantle cell lymphoma includes chemotherapy (using drugs to kill cancer cells), radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) and immunotherapy (using drugs that stimulate the body’s own immune system to kill the cancer cells).

[*]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 459,700,000 (Eurostat 2004).

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There are currently several medicinal products authorised in the Community for treatment of non-Hodgkin lymphomas. The choice of treatment depends in particular on the stage of the disease as well as on the responses to therapies previously prescribed.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that temsirolimus might be of potential significant benefit for the treatment of mantle cell lymphoma, mainly because it may work for patients where other treatments have failed. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Temsirolimus is designed to inhibit (block) the enzyme (a protein that triggers chemical reactions in the body) called serine/threonine kinase. This enzyme plays a role in a cascade of molecular reactions involved in the control of growth and division of cells. In cancer cells, the function of this enzyme is disturbed causing uncontrolled growth and too much division of the cancer cells, making them develop into tumours. According to the sponsor, temsirolimus might, by inhibition of the activity of serine/threonine kinase, help in slowing down or stopping the further growth of cancer cells.Describe as simply as possible the clinically relevant principal mechanism of action and try to link this to the condition applied for.

What is the stage of development of this medicine?

The effects of temsirolimus were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with mantle cell lymphoma were ongoing.

Temsirolimus was not authorised anywhere worldwide for the treatment of mantle cell lymphoma, at the time of submission. Orphan designation of temsirolimus was granted in the European Union for the treatment of renal cell carcinoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 October 2006 recommending the granting of this designation.

Update: temsirolimus (Torisel) has been authorised in the EU since 21 August 2009 for the treatment of adult patients with relapsed and/or refractory mantle cell lymphoma [MCL].

For more information on Torisel, see:
http://www.ema.europa.eu/humandocs/Humans/EPAR/torisel/torisel.htm

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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 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

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.

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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	Temsirolimus	Treatment of mantle cell lymphoma
Bulgarian	Темсиролимус	Лечение на мантелно-клетъчна лимфома
Czech	Temsirolimusum	Léčba lymfomu z plášťové zóny
Danish	Temsirolimus	Behandling af mantelcellelymfom
Dutch	Temsirolimus	Behandeling van mantelcellymfoom
Estonian	Temsirolimus	Mantelrakulise lümfoomi ravi
Finnish	Temsirolimuusi	Manttelisolu-lymfooman hoito
French	Temsirolimus	Traitement des lymphomes du manteau
German	Temsirolimus	Behandlung von Mantelzelllymphom
Greek	Temsirolimus	Θεραπεία του λεμφώματος μανδυκών κυττάρων
Hungarian	Temsirolimus	Köpenysejtes lymphoma kezelése
Italian	Temsirolimus	Trattamento del linfoma con cellule a mantello
Latvian	Temsirolimuss	Mantijšūnu limfomas ārstēšana
Lithuanian	Temsirolimusas	Mantijos ląstelių limfomos gydymas
Maltese	Temsirolimus	Kura tal-limfoma taċ-ċelloli tal-mantell
Polish	Temsirolimus	Leczenie chłoniaków z komórek płaszczowych
Portuguese	Temsirolimus	Tratamento de linfoma de células do manto
Romanian	Temsirolimus	Tratamentul limfomului cu celule în manta
Slovak	Temsirolimus	Liečba lymfómu plášťovej zóny
Slovenian	Temsirolimus	Zdravljenje limfoma plaščnih celic
Spanish	Temsirolimus	Tratamiento del linfoma de células del manto
Swedish	Temsirolimus	Behandling av mantelcellslymfom
Norwegian	Temsirolimus	Behandling av mantelcelle-lymfom
Icelandic	Temsírólímus	Meðferð möttulfrumu eitlakrabbameins

[1] At the time of transfer of sponsorship