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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenalidomide for the treatment of mantle cell lymphoma

On 27 October 2011, orphan designation (EU/3/11/924) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for lenalidomide for the treatment of mantle cell lymphoma.

What is mantle cell lymphoma?

Mantle cell lymphoma is an aggressive cancer of a type of white blood cell called B-lymphocytes, or B cells. In mantle cell lymphoma, the B cells multiply too quickly and live for too long, so there are too many of them in the lymph nodes. The first sign of the disease is usually a lump in the neck, under the arm or in the groin area, caused by an enlarged lymph node. Patients may also have fever, weight loss, tiredness and night sweats.

Mantle cell lymphoma is usually diagnosed in people aged over 50 years. It is more common in men than women. Mantle cell lymphoma is a long-term debilitating and life-threatening disease that is associated with poor overall survival.

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

At the time of designation, mantle cell lymphoma affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 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?

The main treatments for mantle cell lymphoma available at the time of designation do not cure the disease and include chemotherapy (medicines to treat cancer), immunotherapy (medicines that stimulate the body's own immune system to kill the cancer cells) and radiotherapy (treatment with radiation). At the time of designation, temsirolimus was specifically authorised in the EU for the treatment of mantle cell lymphoma that has come back after previous treatment or has not responded

*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 506,300,000 (Eurostat 2011).

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to other treatments. Haematopoietic (blood) stem cell transplantation was also used. This is a complex procedure where patients receive stem cells to help restore the bone marrow.

The sponsor has provided sufficient information to show that lenalidomide might be of significant benefit for patients with mantle cell lymphoma because it works in a different way to existing treatments and might be used in combination with existing methods to improve the treatment of patients. 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?

Lenalidomide is an immunomodulating agent. This means that it affects the activity of the immune system (the body's natural defences). Lenalidomide is expected to work in a number of different ways in mantle cell lymphoma: it blocks the production of cytokines (messenger molecules of the immune system) which help the tumour cells survive, prevents the growth of blood vessels within tumours and also stimulates some of the specialised cells of the immune system to attack the cancerous cells. It also increases production of a protein that blocks an enzyme involved in the control of cell division, to help slow down the growth and spread of the cancer.

What is the stage of development of this medicine?

The effects of lenalidomide have been evaluated in experimental models.

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

At the time of submission, lenalidomide was authorised under the name 'Revlimid' for the treatment of multiple myeloma in the EU and in several countries outside the EU. Outside the EU, lenalidomide was also authorised for the treatment of myelodysplastic syndromes.

At the time of submission, orphan designation of lenalidomide had been granted in the United States for mantle cell lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2011 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	Lenalidomide	Treatment of mantle cell lymphoma
Bulgarian	Lenalidomide	Лечение на мантелно-клетъчна лимфома
Czech	Lenalidomidum	Léčba lymfomu z plášťové zóny
Danish	Lenalidomid	Behandling af mantelcellelymfom
Dutch	Lenalidomide	Behandeling van mantelcellymfoom
Estonian	Lenalidomiid	Mantelrakulise lümfoomi ravi
Finnish	Lenalidomidi	Manttelisolu-lymfooman hoito
French	Lénalidomide	Traitement des lymphomes du manteau
German	Lenalidomid	Behandlung von Mantelzelllymphom
Greek	Λεναλιδομίδη	Θεραπεία του λεμφώματος μανδυκών κυττάρων
Hungarian	Lenalidomid	Köpenysejtes lymphoma kezelése
Italian	Lenalidomide	Trattamento del linfoma con cellule a mantello
Latvian	Lenalidomide	Mantijšūnu limfomas ārstēšana
Lithuanian	Lenalidomidas	Mantijos ląstelių limfomos gydymas
Maltese	Lenalidomide	Kura tal-limfoma taċ-ċelloli tal-mantell
Polish	Lenalidomid	Leczenie chłoniaków z komórek płaszczowych
Portuguese	Lenalidomida	Tratamento de linfoma de células do manto
Romanian	Lenalidomidă	Tratamentul limfomului cu celule în manta
Slovak	Lenalidomid	Liečba lymfómu plášťovej zóny
Slovenian	Lenalidomid	Zdravljenje limfoma plaščnih celic
Spanish	Lenalidomida	Tratamiento del linfoma de células del manto
Swedish	Lenalidomid	Behandling av mantelcellslymfom
Norwegian	Lenalidomid	Behandling av mantelcelle-lymfom
Icelandic	Lenalídómíð	Meðferð möttulfrumu eitlakrabbameins

[1] At the time of designation