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EMA/COMP/210061/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenalidomide for the treatment of marginal zone lymphoma

On 24 April 2015, orphan designation (EU/3/15/1473) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for lenalidomide for the treatment of marginal zone lymphoma.

What is marginal zone lymphoma?

Marginal zone lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In marginal zone lymphoma, abnormal B cells multiply too quickly and live for too long. The abnormal B cells affect various organs. Patients usually have fever, weight loss, tiredness and night sweats.

Marginal zone lymphoma is a life-threatening and long-term debilitating disease due to its effects on the spleen, lymph nodes and bone marrow, as well as the increased risk of infection.

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

At the time of designation, marginal zone lymphoma affected 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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?

At the time of designation, the main treatments for marginal zone lymphoma used in the EU included immunotherapy (using the body's own immune system to kill cancer cells) with the medicine rituximab, chemotherapy (cancer medicines), radiotherapy (treatment with radiation) and surgery to remove affected lymph nodes. In some patients, marginal zone lymphoma affecting the stomach is associated with infection by the bacterium *Helicobacter pylori*, and treatment with antibiotics was used to resolve the infection.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

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The sponsor has provided sufficient information to show that lenalidomide might be of significant benefit for patients with marginal zone lymphoma because early studies in patients show improved effectiveness when the medicine is used together with other medicines authorised for 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?

Lenalidomide is an immunomodulating agent already authorised as Revlimid in the EU for the treatment of multiple myeloma (a cancer of a type of white blood cells called plasma cells) and myelodysplastic syndromes (a group of bone marrow disorders that can cause anaemia). Immunomodulating agents affect the activity of the immune system (the body's natural defences).

Although the exact way lenalidomide works in marginal zone lymphoma is not known, it is thought to work in a number of different ways: it blocks the production of certain cytokines (messenger molecules of the immune system) which help the cancer cells to survive, prevents the growth of blood vessels which provide nutrients to tumours and stimulates some of the specialised cells of the immune system to attack the cancer cells. These actions are expected to slow down the growth and spread of the lymphoma cells.

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 marginal zone lymphoma were ongoing.

At the time of submission, lenalidomide was not authorised anywhere in the EU for marginal zone 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 19 March 2015 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 marginal zone lymphoma
Bulgarian	Леналидомид	Лечение на маргинално зонален лимфом
Croatian	Lenalidomid	Liječenje limfoma marginalne zone
Czech	Lenalidomid	Léčba lymfomu z marginální zóny
Danish	Lenalidomid	Behandling af marginalzonelymfom
Dutch	Lenalidomide	Behandeling van marginale zone lymfoom
Estonian	Lenalidomiid	Marginaaltsooni lümfoomi ravi
Finnish	Lenalidomidi	Marginaalivyöhykkeen lymfooman hoito
French	Lénalidomide	Traitement du lymphome de la zone marginale
German	Lenalidomid	Behandlung des Marginalzonenlymphoms
Greek	Λεναλιδομίδη	Θεραπεία του λεμφώματος μεθοριακής ζώνης
Hungarian	Lenalidomid	Marginális zóna lymphoma kezelése
Italian	Lenalidomide	Trattamento del linfoma della zona marginale
Latvian	Lenalidomīds	Marginālo zonu limfomas ārstēšana
Lithuanian	Lenalidomidas	Marginalinės zonos limfomos gydymas
Maltese	Lenalidomide	Kura ta' limfoma taż-żona marġinali
Polish	Lenalidomid	Leczenie chłoniaka strefy brzeżnej
Portuguese	Lenalidomida	Tratamento do linfoma da zona marginal
Romanian	Lenalidomidă	Tratamentul limfomului de zonă marginală
Slovak	Lenalidomid	Liečba lymfómu z marginálnej zóny
Slovenian	Lenalidomid	Zdravljenje limfoma marginalne cone
Spanish	Lenalidomida	Tratamiento del linfoma de la zona marginal
Swedish	Lenalidomid	Behandling av marginalzonslymfom
Norwegian	Lenalidomid	Behandling av marginalsonelymfom
Icelandic	Lenalídómíð	Meðferð við jaðarsvæðiseitilkrabbameini

[1] At the time of designation