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EMA/COMP/808519/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenalidomide for the treatment of follicular lymphoma

On 24 January 2013, orphan designation (EU/3/12/1097) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for lenalidomide for the treatment of follicular lymphoma.

What is follicular lymphoma?

Follicular lymphoma is a cancer of a type of white blood cell called B-lymphocytes, or B cells. In follicular 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.

Follicular lymphoma is usually diagnosed in people aged over 50 years. It is a life-threatening disease and long-term debilitating due to the cancer coming back, resistance to treatment and organ damage.

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

At the time of designation, follicular lymphoma affected approximately 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 112,000 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 follicular lymphoma available in the EU included chemotherapy (medicines to treat cancer) combined with immunotherapy (medicines that stimulate the body's own immune system to kill the cancer cells). The medicines bendamustine, ibritumomab tiuxetan, interferon alfa 2b and rituximab were specifically authorised for the treatment of follicular lymphoma.

*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 509,000,000 (Eurostat 2013).

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The sponsor has provided sufficient information to show that lenalidomide might be of significant benefit for patients with follicular lymphoma because it works in a different way to existing treatments, and early studies show that when used in combination with existing treatment it might improve the outcome of patients with follicular lymphoma that has come back or has not responded to treatment. 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 authorised for the treatment of multiple myeloma. Immunomodulating agent means that it affects the activity of the immune system (the body's natural defences). Although the exact way lenalidomide works in follicular 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 also stimulates some of the specialised cells of the immune system to attack the cancer cells. This is expected to slow down the growth and spread of the follicular lymphoma.

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 follicular lymphoma were ongoing.

At the time of submission, lenalidomide was not authorised anywhere in the EU for follicular 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 6 December 2012 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 follicular lymphoma
Bulgarian	Леналидомид	Лечение на фоликуларен лимфом
Czech	Lenalidomid	Léčba folikulárního lymfomu
Danish	Lenalidomid	Behandling af follikulært lymfom
Dutch	Lenalidomide	Behandeling van folliculair lymfoom
Estonian	Lenalidomiid	Follikulaarse lümfoomi ravi
Finnish	Lenalidomidi	Follikulaarisen lymfooman hoito
French	Lénalidomide	Traitement des lymphomes folliculaires
German	Lenalidomid	Behandlung des follikulären Lymphoms
Greek	Λεναλιδομίδη	θεραπεία του θηλακιώδους λεμφώματος
Hungarian	Lenalidomid	Follicularis lymphoma kezelése
Italian	Lenalidomide	Trattamento del linfoma follicolare
Latvian	Lenalidomīds	Folikulārās limfomas ārstēšana
Lithuanian	Lenalidomidas	Folikulinės limfomos gydymas
Maltese	Lenalidomide	Kura tal-limfoma follikulari
Polish	Lenalidomid	Leczenie chłoniaków grudkowych
Portuguese	Lenalidomida	Tratamento do linfoma folicular
Romanian	Lenalidomidă	Tratamentul limfomului folicular
Slovak	Lenalidomid	Liečba folikulárneho lymfómu
Slovenian	Lenalidomid	Zdravljenje folikularnega limfoma
Spanish	Lenalidomida	Tratamiento del linfoma folicular
Swedish	Lenalidomid	Behandling av follikulärt lymfom
Norwegian	Lenalidomid	Behandling av follikulært lymfom
Icelandic	Lenalídómíð	Meðferð á follicular eitilfrumukrabbameini

[1] At the time of designation