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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenalidomide for the treatment of diffuse large B-cell lymphoma

On 13 May 2011, orphan designation (EU/3/11/868) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for lenalidomide for the treatment of diffuse large B-cell lymphoma.

What is diffuse large B-cell lymphoma?

Diffuse large B-cell lymphoma is the most common cancer of the lymphatic system, a network of vessels that transport lymph from tissues through the lymph nodes and into the bloodstream. Diffuse large B-cell lymphoma affects a type of white blood cell called B lymphocytes, or B cells. In diffuse large B-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, which is caused by an enlarged lymph node. Patients with diffuse large B-cell lymphoma may also have fever, tiredness, night sweats or weight loss that have no obvious cause.

Although some people with diffuse large B-cell lymphoma can be cured, it remains a serious and life-threatening disease, particularly when the disease is diagnosed late or has come back after initial treatment.

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

At the time of designation, diffuse large B-cell lymphoma affected approximately 1.25 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 63,000 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, several medicines were authorised for the treatment of diffuse large B-cell lymphoma in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem cell

*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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transplantation was also used in patients at risk of the disease coming back 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 lenalidomide might be of significant benefit for patients with diffuse large B-cell lymphoma because early studies show that it might improve the treatment of patients with this condition, particularly in patients whose disease has come back after 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. 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 diffuse large B-cell lymphoma: it blocks the development of tumour cells, 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.

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 including patients with diffuse large B-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, lenalidomide was not authorised anywhere in the EU for diffuse large B-cell 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 9 February 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 diffuse large B-cell lymphoma
Bulgarian	Lenalidomide	Лечение на дифузен В-едроклетъчен лимфом
Czech	Lenalidomidum	Léčba velkobuněčného difuzního B-lymfomu
Danish	Lenalidomid	Behandling af diffust storcellet B-celle lymfom
Dutch	Lenalidomide	Behandeling van diffuus grootcellig B-cel-lymfoom
Estonian	Lenalidomiid	Diffuusse suure β-rakulise lümfoomi ravi
Finnish	Lenalidomidi	Diffuusin suurisoluisen B-solulymfooman hoito
French	Lénalidomide	Traitement du lymphome diffus à grandes cellules B
German	Lenalidomid	Behandlung des diffusen großzelligen B-Zell-Lymphoms
Greek	Λεναλιδομίδη	Θεραπεία του διάχυτου μεγαλοκυτταρικού λεμφώματος Β-κυττάρου (DLBCL)
Hungarian	Lenalidomid	Diffúz nagy B-sejtes lymphoma kezelése
Italian	Lenalidomide	Terapia del Linfoma non-Hodgkin diffuso a grandi cellule di tipo B (DLBCL)
Latvian	Lenalidomide	Difūzas lielo B šūnu limfomas ārstēšana
Lithuanian	Lenalidomidas	Difuzinės stambiųjų B ląstelių limfomos gydymas
Maltese	Lenalidomide	Kura tal-limfoma taċ-ċelluli tat-tip B kbar mxerrda
Polish	Lenalidomid	Leczenie rozlanego chłoniaka z dużych limfocytów B
Portuguese	Lenalidomida	Tratamento do linfoma difuso de grandes células B
Romanian	Lenalidomidă	Tratamentul limfomului difuz cu celule B mari
Slovak	Lenalidomid	Liečba difúzneho veľkobunkového lymfómu z B-buniek
Slovenian	Lenalidomid	Zdravljenje razširjenega limfoma velikih B celic
Spanish	Lenalidomida	Tratamiento del linfoma difuso de células B grandes
Swedish	Lenalidomid	Behandling av diffusa storcelliga B-cells lymfom
Norwegian	Lenalidomid	Behandling av diffust storcellet B-celle lymfom..
Icelandic	Lenalídómíð	Til meðferðar á dreifðu stórfrumu B frumu eitlakrabbameini

[1] At the time of designation